# ANNUAL REPORT

## European Haemophilia Consortium

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

2024

# Table of content

## 01

# Introduction

## Commonly used acronyms

| | | | |
|---|---|---|---|
| **AG** | Activity Grant | **HTC** | Haemophilia Treatment Centre |
| **BAT** | Bleeding Assessment Tool | **ISTH** | International Society on Thrombosis and Haemostasis |
| **CEO** | Chief Executive Officer | **IWG** | Inhibitor Working Group |
| **CAB** | Community Advisory Board | **MAG** | Medical Advisory Group |
| **CMA** | Critical Medicines Alliance | **MASAG** | Medical and Scientific Advisory Group |
| **CoE** | Council of Europe | **MDT** | Multidisciplinary team |
| **EAHAD** | European Association for Haemophilia and Allied Disorders | **MoU** | Memorandum of Understanding |
| **EASL** | European Association for the Study of the Liver | **NMO** | National Member Organisation |
| **EBAH** | European Board of Accreditation in Haematology | **NTR** | Novel Treatment Review |
| **EHA** | European Haematology Association | **OHP** | Organisational Health Programme |
| **EHC** | European Haemophilia Consortium | **PAG** | Physical Activity Grant |
| **EMA** | European Medicines Agency | **PEF** | Patient Education Fund |
| **EP** | European Parliament | **PLUS** | Platform of Plasma Protein Users |
| **ePAG** | European Patient Advocacy Group | **PPR** | Public Policy Review |
| **EPF** | European Patients' Forum | **PROBE** | Patient-Reported Outcomes, Burdens and Experiences |
| **EU** | European Union | **SC** | Steering Committee |
| **EUHASS** | European Haemophilia Safety Surveillance | **UK** | United Kingdom |
| **ERIN** | European Rare and Inhibitor Network | **VRCFD** | Very Rare Coagulation Factor Deficiencies |
| **ERN** | EuroBloodNet - European Reference Network for Haematological Disorders | **VWD** | von Willebrand Disease |
| **EURORDIS** | EURORDIS-Rare Disease Europe | **WBD** | Women and Bleeding Disorders |
| **GA** | General Assembly | **WFH** | World Federation of Hemophilia |
| **HCP** | Healthcare provider | **WG** | Working Group |
| **HTA** | Health Technology Assessment | **WHO** | World Health Organisation |

# Message from the EHC President

*Dear members of the European Haemophilia Consortium (EHC),*

*Reflecting on 2024, it has been a year of transition and consolidation, reaffirming our commitment to National Member Organisations (NMOs). Amid rapid innovation and evolving challenges, we have* ***strengthened our foundation*** *while embracing a* ***modern, patient-centered approach****. However, while we embrace progress, we remain acutely aware of the disparities that persist across Europe. Our mission is to bridge these gaps, ensuring that no one is left behind, regardless of their country.*

This year, we **prioritised direct engagement** through the Patient Education Fund and the country visits, ensuring tailored support for our national communities. We also reinvigorated our European-level advocacy through our round table discussions. These platforms provided an opportunity to address pressing issues, such as treatment inequalities, access to innovative therapies, and the importance of sustainable healthcare policies. We are committed to ensuring that the voices of our community are heard in key policy discussions.

Another key milestone in 2024 was the **establishment of the EHC's three strategic pillars: Education, Communication, and Advocacy** - guiding our efforts to provide resources, foster dialogue, and champion patients' rights. In particular, strengthening our communication efforts will allow us to better engage with our community, share vital information, and amplify the voices of our NMOs. **Fostering partnerships with the World Federation of Hemophilia (WFH)** and the **European Association for Haemophilia and Allied Disorders (EAHAD)** has amplified our collective impact.
As we move forward, ongoing self-assessment is key, especially for our NMOs, to tackle future challenges effectively. Modernisation is essential, but our core philosophy remains: **Patients always come first**. Our diverse community includes people with haemophilia and other bleeding disorders, and our work must be guided by empathy and inclusivity.

We wish to extend our **sincere gratitude** to the **wonderful and professional EHC staff** for their dedication and hard work throughout the year. Their efforts have been instrumental in advancing our mission and supporting our community. Additionally, we express our deep appreciation to the numerous volunteers who generously dedicate their time and energy to the EHC. Their commitment and passion are invaluable, and without them, many of our initiatives would not be possible.

Together, let's **build a stronger, more inclusive, and forward-looking EHC** committed to equal access to care for all.

Miguel Crato

***Miguel Crato***
**EHC President**

# About EHC

**48**

**national patient organisations**

**120,000**

**patients**

The EHC is an international non-profit organisation that **represents 48 national patient organisations** for people with rare bleeding disorders located in the World Health Organisation (WHO) European region, including 27 Member States of the European Union (EU) and most Member States of the Council of Europe (CoE).

The EHC represents approximately 120,000 people diagnosed with rare bleeding conditions such as **haemophilia, von Willebrand Disease (VWD), and other extremely rare bleeding disorders** across Europe. However, experts estimate that many more live with an undiagnosed rare bleeding disorder.

The EHC actively supports its **NMOs at national and European levels** and helps NMOs engage with each other. The EHC draws on the knowledge of patients, healthcare professionals, the scientific community, European institutions, and the pharmaceutical industry to share expertise within Europe. Also, the EHC collaborates closely with other European patient organisations to ensure a strong collective voice for people with rare bleeding disorders.

**Our objectives:**

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

## 02

# Governance

## Steering Committee

The EHC is governed by a General Assembly (GA) composed of representatives from all NMOs. Between general assemblies, the EHC's activities are overseen by a Steering Committee (SC) elected by the GA and composed of the President, Vice President, Treasurer, and Steering Committee members. The SC is elected by the GA for a four-year term.

SC members fulfill their mandate **purely as non-paid volunteers in the best interest of the patient community** they represent and serve. They usually receive no financial gains or compensation for their time and report annually on any private interests they might have.

**Until October 2024, the SC was composed of:**

- **Miguel Crato** | *President*
  Portuguese NMO
- **Jo Eerens** | *Vice President of Finance*
  Belgian NMO
- **Panagiotis Christoforou** | *Co-opted SC Member*
  Greek NMO (since December 2023)
- **Evelyn Grimberg** | *Co-opted SC Member*
  Dutch NMO
- **Diana Lighezan** | *Co-opted SC Member*
  Romanian NMO
- **Admir Mehić** | *Co-opted SC Member*
  Bosnian and Herzegovinian NMO
  (since November 2023)
- **Stefan Radovanović** | *Co-opted SC Member*
  Serbian NMO
- **Olivia Romero Lux** | *Ex-officio Member*
  Chief Executive Officer (CEO) (since April 2024)
- **Luis Teixeira** | *Ex-officio Member*
  CEO (until May 2024)

Following the elections at the 2024 GA, the SC has changed, with **seven new Members elected** — including a new Treasurer and Vice President — and one SC Member co-opted.

**Since October 2024, hence, the SC has been composed of:**


**Miguel Crato**
*President*
Portuguese NMO


**Tatjana Marković**
*Vice President*
Serbian NMO
(4-year term)


**Laura Quintas Lorenzo**
*Treasurer*
Spanish NMO
(2-year term)


**Panagiotis Christoforou**
*SC Member*
Greek NMO
(5-year term)


**William McKeown**
*SC Member*
United Kingdom (UK)
NMO (5-year term)


**Augustas Nedzinskas**
*SC Member*
Lithuanian NMO
(4-year term)


**Jamie O'Hara**
*SC Member*
UK NMO
(5-year term)


**Amy Owen-Wyard**
*SC Member*
UK NMO
(4-year term)


**Marius Tanase**
*Co-opted SC Member*
Romanian NMO
(4-year term)


**Olivia Romero Lux**
*Ex-officio Member*
CEO

## Statutes

To comply with Belgian law, the EHC had to update its statutes and have them **approved under the supervision of a Belgian notary**. The statutes revision was a lengthy process involving all NMOs and was set for approval in June 2024.

Moving forward, there will be **two GAs** per year: one in **June** during the **Leadership Conference** to approve accounts in compliance with the Belgian legislation and one in **October** during the **Annual Conference** to elect the EHC officials and approve the budget.

## Financial Guidelines

The SC drafted and voted on the new financial guidelines to provide an updated and structured framework.

The main objectives of these guidelines are:

- to **successfully monitor** the budgets and consequently, the finances of the organisation;
- to **comply with the rules and regulations** of the Belgian accounting standards;
- to ensure more **transparency and accuracy**.

The guidelines also provide the modalities of attendance to the EHC events for the EHC SC, Medical Advisory Group (MAG), and thematic committees members.

# 03

# Staff

In 2024, the EHC staff underwent significant changes. In March, **Luis Teixeira resigned as CEO** and stayed on to facilitate the handover before departing in May. At the beginning of April, **Olivia Romero Lux** was unanimously elected as the **new CEO** by the SC. After assuming the role of President in an interim caretaking capacity in 2023 and being in the SC for several years, she endorsed her new function with the following priorities: restructure the team, provide a safe and respectful working environment, redefine remits, and hire new staff members.

Throughout the year, other dedicated members of the staff left their positions: Laura Savini resigned as Advocacy Lead in February, followed by Kristine Jansone as Community Development Lead in December.

New talented professionals joined the team: **Camilla Lombardi** as Communication and Project Officer in March; **Roberta Sadauskaitė** as Public Policy Lead in September; **Stéphanie Maya Bravo** as Administration Officer in October, and **Stefan Radovanović** as Patient Engagement and Community Lead in December.

By the end of 2024, then, the EHC team was fully restructured and operational to envision a constructive and fruitful year 2025.

In May, **Fiona Brennan** transitioned from her role as Patient Engagement Lead to Special Advisor. Since then, she has been supporting the CEO in managing all interactions between patients and industry—such as Community Advisory Boards (CAB) and specific meetings—as well as assisting other staff members in programme development and facilitating select EHC events.

At the end of 2024, the EHC officially launched an internship programme, opening a new exciting chapter in nurturing talent and fostering growth within the organisation. Typically lasting six months, the EHC internship offers young individuals a chance to work with our dynamic, international team based in Brussels.

In December 2024, the EHC welcomed **Stavroula Koukouzeli** as Project Assistant under the supervision of Zita Gacser for the next six months.

## 04

# Membership

**In 2024, the following organisations were part of the EHC membership:**

| | | | |
|---|---|---|---|
| **Albania** | Shoqatës Shqiptare të Hemofilikëve | **Latvia** | Latvijas Hemofilijas Biedrība |
| **Armenia** | Armenian Association of Hemophiliacs | **Lithuania** | Lietuvos Žmonių Sergančių Hemofilija Asociacija |
| **Austria** | Österreichische Hämophilie Gesellschaft (ÖHG) | **Luxembourg** | Association Luxembourgeoise des Hémophiles |
| **Azerbaijan** | Hemofiliyalı Xəstələrin Respublika Assosiasiyası | **Malta** | Malta Bleeding Disorders Society |
| **Belarus** | Белорусская Ассоциация Больных Гемофилией | **North Macedonia** | Hemolog |
| **Belgium** | Association de l'hémophilie, von Willebrand et autres pathologies de la coagulation / Vereniging van hemofilie-, von Willebrandpatiënten en andere Stollingsstoornissen (AHVH) | **Moldova** | Ассоциация Гемофилии Молдовы |
| **Bosnia and Herzegovina** | Udruženje hemofiličara Bosne i Hercegovine | **Montenegro** | Crnogorsko društvo za hemofiliju |
| **Bulgaria** | Българска Асоциация по Хемофилия | **Netherlands, the** | NVHP voor iedereen met een erfelijke stollingsstoornis (NVHP) |
| **Croatia** | Društvo hemofiličara Hrvatske | **Norway** | Foreningen for blødere i Norge (FBIN) |
| **Cyprus** | Παγκύπρια Οργάνωση Αιμορροφιλικών (Π.Ο.Α.) | **Poland** | Polskie Stowarzyszenie Chorych na Hemofilię |
| **Czechia** | Český svaz hemofiliků | **Portugal** | Associação Portuguesa de Hemofilia e de outras Coagulopatias Congénitas (APH) |

| | | | |
|---|---|---|---|
| **Denmark** | Bløderforening | **Romania** | Asociația Română de Hemofilie (ARH) |
| **Estonia** | Eesti Hemofiiliaühing | **Russia** | Всероссийское общество гемофилии |
| **Finland** | Verenvuotosairaudet ry | **Serbia** | Udruženje hemofiličara Srbije |
| **France** | Association Française des Hémophiles (AFH) | **Slovakia** | Slovenské hemofilické združenie |
| **Georgia** | Georgian Association of Hemophilia and Donorship | **Slovenia** | Društvo hemofilikov in drugih z motnjami strjevanja krvi Slovenije |
| **Germany** | Deutsche Hämophilie Gesellschaft | **Spain** | Federación Española de Hemofilia (FEDHEMO) |
| **Greece** | Σύλλογος Προστασίας Ελλήνων Αιμορροφιλικών (ΣΠΕΑ) | **Sweden** | Förbundet Blödarsjuka i Sverige |
| **Hungary** | Magyar Hemofília Egyesület | **Switzerland** | Schweizerische Hämophilie Gesellschaft |
| **Iceland** | Blæðarafélag Íslands | **Tajikistan** | Общественная организация «Гемофилия» |
| **Ireland** | Irish Haemophilia Society | **Türkiye** | Türkiye Hemofili Derneği |
| **Israel** | המופיליה לחולי עמותה – עלה | **Ukraine** | Всеукраїнське товариство Гемофілії |
| **Italy** | Federazione delle Associazioni Emofilici (Fedemo) | **United Kingdom, the** | The Haemophilia Society |
| **Kyrgyzstan** | Общество инвалидов-больных гемофилией Кыргызской Республики | **Uzbekistan** | Узбекское общество гемофилии |

# Grants

**€49.969,33**

**total funding**

**9**

**countries received grants**

In 2024, the EHC embedded **Activity Grants (AGs)** and **Physical Activity Grants (PAGs)** into the newly developed Patient Education Fund (find more information on page 42).

These grants **help NMOs initiate or sustain projects** that improve the quality of life for people with rare bleeding disorders. AGs support a wide range of initiatives, while PAGs specifically fund activities that enhance the physical capacity of individuals living with these conditions.

In 2024, the EHC awarded the following AGs and PAGs, providing a total of **€49.969,33** in funding.

The **AGs** supported a range of initiatives, including the organisation of an NMO annual meeting, a Therapeutic Innovation Day in Réunion Island (France), the translation of EHC materials, collaboration with Healthcare providers (HCPs) on a nationwide patient screening, and a members' camp.

Additionally, **PAGs** funded various activities such as Nordic walking, indoor exercises, pool exercises, and a basketball camp.

These initiatives played a key role in **strengthening NMO activities and advancing the EHC's mission**.

**Activity Grants**

- Bosnia and Herzegovina
- Finland
- France
- Israel
- Sweden
- United Kingdom

**Physical Activity Grants**

- Croatia
- Lithuania
- Slovenia

# 05

# 2024 – 2027 Strategic Plan

After the **EHC Strategic Retreat** held in January 2024, where all SC, Staff, MAG members, and committee representatives were invited to participate, a Strategic Plan for 2024-2027 was drafted. The plan was then approved by the SC and presented to NMOs during the Leadership Conference in June.

**The 2024-2027 Strategic Plan is based on the following three pillars:**

## Advocacy

- Focus on **NMOs** and **country visits**
- **Inclusivity** and information dissemination
- **External presence**

## Communication

- **Internal** communication **improvement**
- **External** communication **enhancement**

## Education

- Focus on **multidisciplinary care**
- **Standards of care** for other bleeding disorders
- Education **follow-up**

# 06

# Focus areas

The EHC bleeding disorders community represents individuals with various rare congenital bleeding disorders **across 48 EHC NMOs**. Committed to a multidisciplinary approach, the EHC addresses the complex challenges faced by people living with these conditions—**not only the disease itself, including symptoms, severity, and diagnosis, but also critical aspects such as gender, youth, ageing, and both mental and physical health**.

To advance its work in key areas, the EHC has established dedicated working groups and committees that lead efforts in selected focus areas.

## Medical Advisory Group (MAG)

The MAG is a group of experts providing high-level medical and scientific support to the EHC.

They offer strategic input to the SC and staff, and are consulted on ad hoc matters, serving as a valued source of expertise and guidance.

In 2024, the MAG was composed of:

- **Dr Maria Elisa Mancuso** (Chair) | Italy
- **Prof Jan Blatný** | Czechia
- **Prof Ana Boban** | Croatia
- **Prof Johannes Oldenburg** | Germany

## Medical And Scientific Advisory Group (MASAG)

As of October 2024, the MASAG has been discontinued. Prior to this, the Advisory Group brought together a **diverse team of medical and scientific experts**, offering the EHC valuable advice on specific activities with a multidisciplinary approach. It also provided reliable expertise on legislative matters, supporting key areas of EHC's work.

The EHC wishes to thank all former MASAG members for their unwavering support over the years.

## Novel Treatment Review (NTR) Working Group (WG)

The NTR WG oversees the production of the NTR, a periodic review of novel treatments in haemophilia and other bleeding disorders, by supporting the EHC staff in developing its content. In 2024, the EHC published two NTR editions (find more information on page 25).

In 2024, the NTR WG was composed of:

- **Paul Batty** | University College London | UK
- **Ana Boban** | MAG | Croatia (since October 2023)
- **Mariëtte Driessens** | Dutch NMO
- **Miguel Crato** | EHC President | Portuguese NMO (since October 2023)
- **Radoslaw Kaczmarek** | Polish NMO
- **Ilmar Kruis** | Dutch NMO
- **Maria Elisa Mancuso** | MAG | Italy (since October 2023)
- **Brian O'Mahony** | Irish NMO
- **David Page** | Canadian Haemophilia Society
- **Uwe Schlenkrich** | Germany
- **Suthesh Sivalaparatnam** | Queen Mary University of London | UK (until November 2024)

# European Rare and Inhibitor Network (ERIN) Committee

ERIN was **created in 2022** with the mission to **expand the EHC Inhibitor Working Group (IWG) to advocate for better inclusion** of people with extremely rare bleeding disorders. Since then, the ERIN Committee has been responsible for developing, approving, and helping to implement the working programme of the Network. The Committee consists of people affected by extremely rare bleeding disorders, people affected by haemophilia A and B with inhibitors, as well as MDT experts.

In 2024, the Committee continued its efforts to raise the visibility of people with extremely rare bleeding disorders by publishing **podcasts featuring patient stories** and **creating content for the ERIN-focused sessions at EHC events**. It also organises the **annual ERIN Summit** (find more information on page 38) and leads the **EHC awareness campaign** for Rare Disease Day (find more information on page 31).

In 2024, the ERIN Committee was composed of:

- **Maja Søndergaard Knudsen** (Chair) | Danish NMO
- **Enea Atroce** | Swiss NMO
- **Christina Burgess** | UK
- **Dominik Čepić** | Croatian NMO
- **Maria Elisa Mancuso** | Italy
- **Jim O'Leary** | Irish NMO
- **Amy Owen-Wyard** | SC Liaison

The Committee's work was supported by **Fiona Brennan** until December 2024, when **Stefan Radovanović** assumed the role of EHC Patient Engagement and Community Lead.

**EHC Community website**

To get in touch with the ERIN Committee: erin@ehc.eu

To **learn more** about the EHC's work in this focus area, please visit the dedicated page on the **EHC Community website**.

# Von Willebrand Disease (VWD) Committee

In 2024, the VWD Committee continued its efforts to strengthen the European VWD community through the **#VWDunited initiative**.

Following the addition of new members in December 2023, the Committee collaborated to develop fresh ideas for upcoming projects, which were further refined during their **first in-person meeting in Madrid in April 2024**.

The Committee remained focused on the three key areas previously agreed upon: **awareness**; **engagement**; and **education & research**.

It is also responsible for organising the EHC awareness campaign for European VWD Awareness Day (find more information on page 30).

In 2024, the VWD Committee was composed of:

- **Julia Rauscher** (Co-chair) | Austrian NMO
- **Joanne Traunter** (Co-chair) | UK NMO
- **Annabel Kattai** | Estonian NMO (until July 2024)
- **Başak Koç Şenol** | Turkish NMO (since October 2024)
- **Laura Quintas Lorenzo** | SC Liaison
- **Cathy Verbraeken** | Dutch NMO
- **Baiba Ziemele** | Latvian NMO

In 2024, SC members **Olivia Romero Lux**, **Admir Mehić**, and **Laura Quintas Lorenzo** supported the Committee's work. EHC Communications and Education Manager **Daria Camilli** assisted the Committee until the end of the year, when EHC Behavioural Change Lead **Zita Gacser** took over.

**EHC Community website**

To get in touch with the **EHC VWD Committee**: vwd@ehc.eu

Reach out to us if you want to **join our WhatsApp support group** (open to VWD patients only!).

To **learn more** about the EHC's work in this focus area, please visit the dedicated page on the **EHC Community website**.

# Women and Bleeding Disorders (WBD) Committee

The WBD Committee aims to improve the quality of life for women and girls impacted by bleeding disorders. At the European level, it focuses on **raising awareness, recognition, support, and education** for women in the bleeding disorder community. The Committee is also responsible for organising the EHC **awareness campaign for International Women's Day** (find more information on page 32).

In 2024, the Committee was represented at the EHC Strategic Retreat in January in Brussels, where they aligned on priorities and collaborated with other EHC Committees members, the Steering Committee, and staff. In April, several members participated in the WFH Congress in Madrid, contributing to global discussions on haemophilia and related disorders.

Their engagement continued at the EHC Leadership Conference in June in Brussels, Belgium, where they supported leadership development across the community. In October, they hosted an interactive lunch break session at the EHC Annual Conference in Sofia, Bulgaria, using Mentimeter for dynamic audience engagement. The year concluded with a Strategic Retreat in Brussels at the end of October, where the Committee focused on strengthening internal dynamics and refining future strategies.

In 2024, the WBD Committee was composed of:

- **Anna Tollwé** (Chair) | Swedish NMO
- **Marion Bräuer** | Austrian NMO
- **Yannick Collé** | French NMO
- **Karola Diósi** | Hungarian NMO (until November 2024)
- **Jo Eerens** | SC Liaison (until October 2024)
- **Tatjana Marković** | SC Liaison (since October 2024)
- **Ana Cristina Monteiro Pastor** | Portuguese NMO
- **Bernadetta Pieczynska** | Polish NMO (since May 2024)
- **Brina Zaman** | Slovenian NMO (since May 2024)

The Committee's work was supported by EHC Behavioural Change Lead **Zita Gacser**.

EHC Community website

To **learn more** about the EHC's work in this focus area, please visit the dedicated page on the **EHC Community website**.

# Youth Committee

The Youth Committee provides **strategic direction** for EHC youth activities, like workshops, fellowships, youth support, and youth-oriented sessions at EHC events. It is also responsible for **designing and managing the agendas and content of youth training and events**, such as the Youth Leadership Workshop and Youth Alumni Events, while overseeing the EHC Youth Fellowship Programme.

In April 2024, the Committee organised the **annual Youth Leadership Workshop in Brussels**, Belgium, with a programme tailored to the interests and needs of young people (find more information on page 36). Later, in October 2024, during the EHC Conference, the Committee hosted its **annual Youth Debate session**, providing young members with a platform to showcase their public speaking skills and apply the insights gained from the Workshop.

In 2024, the Youth Committee was composed of:

- **Enea Atroce** | Swiss NMO
- **Lorenzo Ghirardi** | Italian NMO
- **Zsófia Keszthelyi** | Hungarian NMO
- **Admir Mehić** | SC Liaison (until October 2024)
- **William McKeown** | SC Liaison (from October 2024)
- **Stefan Radovanović** | EHC Youth Advisor | Serbian NMO (until December 2024)

The Committee's work was supported by **Fiona Brennan** until December 2024, when **Stefan Radovanović** assumed the role of EHC Patient Engagement and Community Lead.

EHC Community website

To **learn more** about the EHC's work in this focus area, please visit the dedicated page on the **EHC Community website**.

## 07

# 2024 Activities

# Advocacy

## Novel Treatment Review (NTR)

The NTR is a **periodic review of novel treatments in haemophilia and other bleeding disorders** entirely developed by the EHC to help educate EHC NMOs about the rapidly changing landscape of medicinal treatment in rare bleeding disorders.

The review covers recent major developments and is divided by different types of disorders for which there is an update to report. The information provided in this publication is compiled from multiple sources, including presentations at recent scientific meetings, websites, financial information, and writing directly to pharmaceutical companies. It is then redrafted and presented in easy-to-understand language.

This review is updated twice a year. The EHC encourages its NMOs to use and adapt this document to their national needs, but does not bear responsibility for any modifications made to its content.

This document is overseen by the NTR WG and edited by both NTR WG volunteers selected members of the EHC MAG.

In 2024, the EHC published two editions.

EHC Academy

All **NTR issues** are available on the **EHC Academy**.

## Public Policy Review (PPR)

The PPR of EU legislation is developed entirely by the EHC to inform its NMOs about **health policy and legislative developments** that directly impact the bleeding disorders community.

Initially shared as a quarterly policy review, it has now been transformed into a periodic report available on the EHC Academy.

In January 2024, the EHC published a PPR covering legislative initiatives from the second half of 2023. However, due to understaffing, no additional issues were released in 2024, with the plan to resume publication in early 2025 following staff expansion.

EHC Academy

All **PPR issues** are available on the **EHC Academy**.

## Round Tables

The EHC hosts a series of Round Tables that **bring together stakeholders in the field of congenital bleeding disorders** around one table. The main objective is to provide a neutral forum for constructive and open education, discussion, and exchange on central issues. In addition to industry and patient stakeholders, the Round Tables target key European policy and decision-makers.

In 2024, the EHC held two round tables, **one in September and other one in December**. Due to the European Parliament (EP) elections, the EHC organised both 2024 Round Tables at the Warwick Hotel instead of the European Parliament in Brussels - with the plan to return to the EP in 2025. Find more information on page 39.

## Think Tank

Launched in 2021, the EHC Think Tank serves as a **platform for driving healthcare system change** by identifying key areas for transformation and addressing complex issues through broad, multi-stakeholder collaboration, ultimately enhancing patients' quality of life. Over three years, it made significant strides in shaping the future of healthcare in Europe across five thematic workstreams: **Registries, Hub & Spoke Model** (which was discontinued in February 2023), Patient Agency, Access Equity, and Future Care Pathways.

In 2024, the Think Tank entered a new phase, building on the valuable learnings and outcomes from its workstreams. By 2025, while the Think Tank in its original form has concluded, the EHC advocacy will focus on implementing these insights. This includes **active engagement with decision-makers** at the EU and CoE institutions, as well as at the national level through EHC NMOs.

**We extend our heartfelt appreciation to the workstream members**, whose dedication, expertise, and collaborative spirit were pivotal to the EHC Think Tank's achievements.

EHC Think Tank

Find more information on the **EHC Think Tank website**.

### Publications

Gacser Z., Skouw-Rasmussen N.; *EHC Think Tank. System change in practice: A report from the EHC Think Tank workstreams on Registries and Patient Agency.* J Haem Pract 2024; 11(1): 1-10.

Gacser Z., Bourke S., Hosszú D., Daniels S.; *EHC Think Tank. System change in practice: A report from the EHC Think Tank workstreams on Access Equity and Future Care Pathways.* J Haem Pract 2024; 11(1): 99-107.

*Guidebook on Challenges and Interventions Around Patient Agency.*

## Collaborations and Partnerships

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

**ERN-EuroBloodNet**

**European Association for Haemophilia and Allied Disorders (EAHAD)**

**European Association for the Study of the Liver (EASL)**

**European Hematology Association (EHA)**

**European Patients' Forum (EPF)**

**EURORDIS-Rare Diseases Europe**

**International Society on Thrombosis and Haemostasis (ISTH)**

**Platform of Plasma Protein Users (PLUS)**

**Transform Alliance**

**World Federation of Hemophilia (WFH)**

**Critical Medicines Alliance (CMA)**

In 2024, the EHC decided to streamline and centralise its **representation in third parties' boards, committees, European Patient Advocacy Groups (ePAGs) and working groups.** The EHC resumed participating in many events organised by multi-stakeholders: CEO Olivia Romero Lux, Communications and Education Manager Daria Camilli and Public Policy Lead Roberta Sadauskaite attended many meetings.

The **WFH Medical Advisory Board** met four times in 2024 (March, June, September, and December), and the EHC was represented at all meetings by EHC CEO Olivia Romero Lux. On 12 December, the EHC SC nominated Panagiotis Christoforou to replace her.

The **WFH Coagulation Product Safety, Supply, and Access (CPSSA) Committee** — where EHC President Miguel Crato serves as a representative — met three times in 2024 to discuss and align on common global positions regarding the safety, supply, and accessibility of haemophilia treatments.

Gaëtan Duport played a pivotal role in representing the EHC at the **European Commission Health Technology Assessment (HTA) Stakeholders meetings**, especially with the vote and implementation of several key European legislations in 2024 and 2025. In October 2024, EHC Public Policy Lead Roberta Sadauskaite also joined the stakeholder group.

Baiba Ziemele represented the EHC at the ePAG for ERN-EuroBloodNet, participating in 24 meetings and contributing to key initiatives. She presented the EHC-ERN-EuroBloodNet Focus on Topic: VWD Educational Programme at the 6th ERN-EuroBloodNet Progress Meeting (find more information on page 35), and participated in the European Rare Blood Disorders Platform (ENROL) and Data Access Committee (DAC) discussions on electronic health data regulation.

Additionally, she represented the EHC at the **ePAG for the European** Commission's Workshop on patient rights in cross-border healthcare, worked on EURORDIS Rare Barometer surveys, and contributed to the JARDIN project on ERN integration into national healthcare systems. On 12 December, the EHC SC nominated Marius Tanase to replace Baiba Ziemele at the ePAG for ERN-EuroBloodNet.

In February 2024, the EHC signed a **Memorandum of Understanding (MoU)** with EAHAD to strengthen collaboration. In June, Laura Quintas Lorenzo joined the **EAHAD Women and Girls with Bleeding Disorders Working Group**, which worked on the booklet *Time to Talk: Period* to be translated into several languages. The Working Group focused its activities on developing resources on prophylaxis for heavy menstrual bleeding. Amy Owen-Wyard represented the EHC at the EAHAD Glanzmann Thrombasthenia Working Group which convened twice in 2024. As part of its ongoing efforts, the Working Group drafted three posters for submission to the EAHAD Congress 2025. As member of the EAHAD Gene Therapy Working Group, EHC President Miguel Crato co-authored two important publications issued in *Haemophilia*, the official journal of the WFH—one publication on the role of multidisciplinary teams (MDTs), and another on regional differences in hub-and-spoke models. The Working Group's efforts also focused on developing a standardised lexicon, a gene therapy trial database and strengthening collaboration with hepatologists on liver-related considerations.

Additionally, a webinar on shared decision-making for gene therapy was organised to highlight the importance of patient-centred treatment choices. Besides, Miguel Crato and Panagiotis Christoforou represented the EHC in the EAHAD Accreditation and Audit of Haemophilia Centres Working Group, which held five meetings in 2024 to assess applications for certification as European Haemophilia Centres or Comprehensive Centres. EAHAD and EHC awarded certification to 10 centres. As part of the process, Panagiotis Christoforou participated in a full-day training with Kerteza in September, alongside healthcare professionals, physiotherapists, social workers, and patient representatives, to develop auditing skills based on predefined guidelines. The pilot phase launched with an onsite audit in Frankfurt on 5 November, where auditors conducted a comprehensive evaluation. Insights from Frankfurt will help refine future audits and shape the long-term strategy for EAHAD certification.

Maja Søndergaard Knudsen represented the EHC in the newly established EAHAD Very Rare Coagulation Factor Deficiencies (VRCFD) Working Group, which held three online meetings in 2024. As part of its short-term projects, the Working Group launched e-learning modules on Factor XI Deficiency and Congenital Fibrinogen Disorder. Additionally, throughout 2024, it developed two VRCFD-related surveys, set to be published in 2025—one for Haemophilia Treatment Centres (HTCs) on their patients with VRCFD, and another for patients themselves.

Radoslaw Kaczmarek represented the EHC at the European Haemophilia Safety Surveillance (EUHASS), a pharmacovigilance programme to monitor the safety of treatments for people with inherited bleeding disorders in Europe. He was replaced by William McKeown on 1 December 2024.

To ensure the continued presence of the EHC within the **ePAG for EHA**, Jo Eerens has replaced Manon Degenaar-Dujardin, who represented the EHC in the development of the Bleeding Assessment Tool (BAT) edited by Prof Rodeghiero. In 2024, Jo Eerens participated in the annual meeting in the Hague, where, alongside fellow ePAG members, he contributed to establishing the group's rules and statutes. He also attended the EHA 2024 Congress in Madrid.

In April 2024, the EHC joined the Critical Medicines Alliance (CMA) to work together with over 250 different groups from the public and private sectors to ensure the availability of critical medicines for patients in Europe. The CMA is a new consultative mechanism bringing together national authorities, industry, civil society, the European Commission, and EU agencies to identify the best measures to address and avoid shortages of critical medicines. The CMA's work focuses on critical medicines facing the greatest vulnerabilities, as outlined in the EU List of Critical Medicines published by the European Medicines Agency (EMA) in December 2023. Since von Willebrand factor is not currently included, the EHC has been advocating for its addition in the next edition, scheduled for release by the end of 2025.

At the beginning of 2024, Laura Quintas Lorenzo and Panagiotis Christoforou represented the EHC as expert evaluators in EMA meetings.

In May 2024, Mariëtte Driessens was appointed as a patient representative to the EMA's Committee for Orphan Medicinal Products (COMP), with the support of the EHC. Her three-year term began in July 2024.

# Awareness Campaigns

## European VWD Awareness Day

1 February 2024

On February 1, the second European VWD Awareness Day was marked across Europe. On this special day—the birthday of Erik Adolf von Willebrand—the EHC VWD Committee unveiled the new brand identity of the European VWD Community: a logo and the motto **Making the invisible visible**, reminding community and supporters to use the VWD social media hashtags **#VWDunited #VWDtogethere #beVWDaware**.

Since its founding in 2020, the VWD Committee has worked to build a united European community for those affected by VWD. Its goal has been to **provide support and a strong identity for the community, while developing a network of advocates** across Europe to improve diagnosis, treatment options, and overall care at both national and European levels.

Furthermore, on European VWD Awareness Day, the EHC and ERN-EuroBloodNet launched the online educational programme *Topic on Focus: Von Willebrand Disease (VWD)* for patient organisations and healthcare professionals to be carried out throughout the year (find more information on page 35).

EHC Website

To learn more about the campaign, please visit the **EHC website**.

### The VWD Committee Co-chairs' Statements for the campaign:

> "The VWD committee is celebrating a fruitful and industrious year. Its bruise **campaign has highlighted the consequences of living with VWD**, an often unrecognised and underrepresented condition. We have chosen the image of the heart tattoo from our bruise campaign to represent the work of the committee and our tireless work to support the VWD community.
>
> Our slogan, '**Making the Invisible Visible**' is further symbolic of the campaign to raise awareness and educate those who encounter persons living with the disease whilst supporting those who are still fighting for diagnosis and treatment. Our work does not end; together we are working to change the landscape, to raise awareness and to ensure that all individuals from our VWD community across Europe gain the recognition, support and treatment they deserve."

**Jo Traunter,**
**EHC VWD Committee Co-chair**

> "There are 47 countries in Europe, which also means that there are just as many varying healthcare systems. Each of them runs differently, but no matter how well-developed some countries may be, there are still **a lot of VWD patients all over Europe who struggle to get the medical attention they need** or are even undiagnosed.
>
> That's why we, as the EHC VWD Committee, want to put **the focus on 'Making the invisible visible' on this year's VWD Awareness Day**. We have been working very hard for the last four years to push forward the needs of the patients within the VWD community on a European level, and, finally, our visions and projects start to become reality."

**Julia Rauscher,**
**EHC VWD Committee Co-chair**

## Rare Disease Day

29 February 2024

Rare Disease Day, marked on **29 February** in **leap years** and **28 February in common years**, is the rarest day of the year—fittingly dedicated to raising awareness of rare diseases and **advocating for better access to treatment and medical representation**. For the EHC community, Rare Disease Day is an opportunity to highlight the unique challenges faced by individuals with extremely rare bleeding disorders, including those with haemophilia and inhibitors, as well as their families. The goal is to **broaden the conversation to include those who have often been overlooked**—particularly individuals still struggling to receive an accurate diagnosis or any diagnosis at all. To mark Rare Disease Day on 29 February 2024, the EHC ERIN Committee joined **EURORDIS's awareness campaign** to spotlight the realities of living with extremely rare bleeding disorders. The campaign aimed to not only inform the general public but also address the most pressing issues faced by patients, helping to pave the way for improved treatment options and a better quality of life.

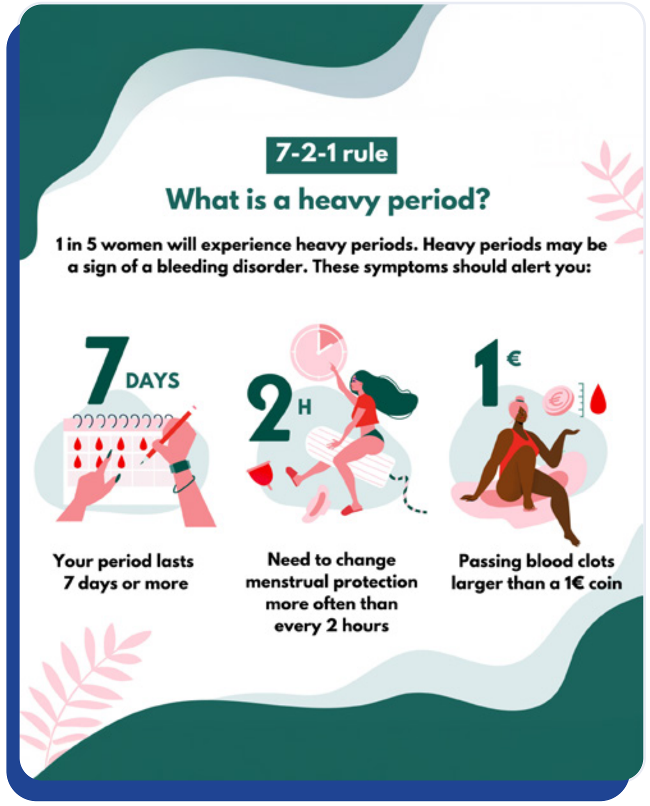
As part of these efforts, the Committee hosted an **extremely rare bleeding disorder webinar**, bringing together healthcare professionals, patients, caregivers, EHC NMO representatives, and the broader community. The event focused on sharing experiences, best practices, and challenges within the extremely rare bleeding disorder community. Key themes included the need for greater awareness and education, fair representation, and stronger collaboration with HCPs and HTCs.

EHC Website

To learn more about the campaign, please visit the **EHC website**.

## International Women's Day

8 March 2024



Each year, the EHC WBD Committee marks International Women's Day with a dedicated awareness campaign. On 8 March 2024, the Committee launched the **7-2-1 Rule infographic in 11 languages to help recognise heavy menstrual bleeding** as a key symptom of rare bleeding disorders.

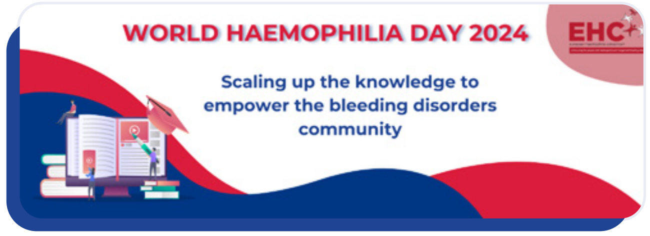
The campaign also featured a customisable template freely available for EHC NMOs to be adapted to their local needs. Through this initiative, the Committee continued to advocate for early recognition, timely diagnosis, and improved care for women and girls affected by bleeding disorders.

**EHC Community website**

To download the 7-2-1 rule infographic, please visit the **EHC Community website**.

## World Haemophilia Day

17 April 2024



Every year, on April 17, the bleeding disorders community worldwide marks World Haemophilia Day to raise **awareness about all bleeding disorders** and advocate for better access to treatment and care.

In 2024, the EHC's focus shifted to education, aiming to empower the bleeding disorders community through knowledge. As part of this effort, the EHC launched the *EHC Academy* was launched — a comprehensive educational platform offering valuable resources on bleeding disorders.

The platform is organised into four key sections: Treatment, Legislation, Access to Treatment and Care, and Advocacy Materials.

Through unique, high-quality knowledge and practical tools, the EHC aimed to elevate advocacy skills and pave the way toward a better quality of life for patients across Europe.

**EHC Website**

To learn more about the campaign, please visit the **EHC website**.

## #ThisWay Campaign

Physical activity continues to play a vital role in managing bleeding disorders — strengthening muscles and joints, reducing the risk of bleeds, and enhancing overall quality of life. In 2024, the EHC's #ThisWay campaign stayed true to its mission of **promoting health and well-being** through patient stories, physical activity initiatives, and community engagement — proving that every step matters.

Over the year, the EHC spotlighted **three powerful patient stories**, offering insight into the varied experiences of people living with bleeding disorders, as well as the challenges faced by caregivers. These personal accounts served to inspire, educate, and empower the community, highlighting the value of resilience, advocacy, and self-care.

- Annabel's story (patient with von Willebrand Disease, Estonia)
- Camilla's story (caregiver, Switzerland/Sweden)
- Adam's story (patient with severe haemophilia B, Poland)

The campaign also hosted two engaging physical activity challenges, **motivating participants to stay active and prioritise their health**. The 2024 Spring Challenge, launched on 25 March and running for four weeks, invited participants to track a wide range of activities — from walking and cycling to Pilates, meditation, and even gardening. This holistic approach underscored the importance of both physical and mental well-being, with prizes awarded to the top three participants who logged the most workouts.

Later in the year, the 2024 Autumn Challenge built momentum in the lead-up to the EHC Annual Conference in Sofia, Bulgaria. What began as a collective goal to reach 200 km quickly turned into an inspiring display of community spirit: the target was surpassed within four days. The bar was then raised to 1.000 km — and participants met it enthusiastically, achieving **an impressive final total of 1.011,73 km in just two weeks**. All contributors received certificates of participation, with the top three recognised with gifts for their exceptional effort.

**EHC Community website**

To learn more about the campaign, please visit the **EHC Community website**.

# Courses and programmes

## Health Economics Course

16 September - 31 October 2024

**100%**

**Overall rate of the course (50% excellent, 30% very good, 20% good)**

Following the successful launch of the EHC Health Economics Course in 2023, **a second online edition** was offered in 2024 to continue providing accessible education for patients and patient organisations on demand for engaging in HTA.

The course featured subtitled **online educational modules** covering **key health economics concepts** relevant to bleeding disorders, including HTA, cost-effectiveness, budget impact models, alternative payment approaches, and treatment updates. Each module included review questions, with additional materials provided for further exploration.

Participants who passed the final quiz could download a certificate of completion and share it on social media.

Building on positive feedback from the first edition, the 2024 version retained its core programme while providing content updates and introducing enhancements for a better learning experience. These included a new platform and the replacement of Q&A sessions with a dedicated group where **participants could interact directly with course experts**.

> "I really appreciated the opportunity to learn about health economics in a way that kept me engaged with the quizzes and a way to test my knowledge and go back to fill any gaps missing.
>
> Having a month to complete the course allowed me to go at my own pace, and I never felt rushed. I genuinely look forward to more opportunities like this and will definitely keep an eye out for future courses."

> "I was really surprised by how much I enjoyed this course. It opened my eyes to aspects of health economics I didn't even know I was missing. I realized there's so much more to learn, and I'm looking at this course as a stepping stone for further research."

> "It was valuable to me to support the patient group that I am involved with. Understanding how the HE process works will give me the tools to understand their needs better."

The EHC wishes to thank the Prime Global (HCD Economics) team, EHC MAG Chair Maria Elisa Mancuso, and Brenda Padilla for their invaluable input in the content of this course. As we express our gratitude, we also want to acknowledge the efforts of the lecturers who have worked to ensure the success of the course: Jonathan Evans, Enrico Ferri Grazzi, Idaira Rodriguez Santana, and Claudia Mighiu.

## EHC – ERN-EuroBloodNet *Topic on Focus: VWD* Educational Programme

1 February 2024 - 20 March 2025

The EHC and **ERN-EuroBloodNet** launched the *Topic on Focus: VWD for Patient Organisations and Healthcare Professionals* on European VWD Awareness Day 2024 (1 February) to run until 20 March 2025.

This educational initiative was developed in partnership with **EURORDIS-Rare Diseases Europe** and supported by members of the scientific committee: Prof Sophie Susen (CHU de Lille, France), Prof Jeroen Eikenboom (Leiden University Medical Centre, the Netherlands), and Prof Flora Peyvandi (Foundation IRCCS Ca'Granda Ospedale Maggiore Policlinico, Milan, Italy). The programme was also accredited by the European Board of Accreditation in Haematology.

This year-long project comprised expert-led webinars addressing crucial topics related to VWD offering valuable insights for both patients and HCPs, while shedding light on the challenges faced by those living with this rare disease.

The Programme covered the following topics:

- VWD is not haemophilia
- Genetics of VWD
- Type doesn't define VWD symptoms
- People with VWD bleed too
- Do not let people bleed their way to treatment: accurately diagnosing VWD
- Treating VWD: What are the options?
- Surgery
- Women bleed too (menstruation, managing periods, impact, sex with VWD, etc.)
- Maternity
- Ageing
- VWD in the emergency room
- Emotional trauma
- Improving the quality of life for VWD patients

The **webinar recordings** are available to the public on the EHC YouTube channel (@EHCTVChannel). These resources are designed for patients, caregivers, HCPs, and anyone interested in learning more about VWD.

## Organisational Health Programme (OHP)

Approved by EHC NMOs in 2022, the OHP has been part of the EHC's work since 2023, offering its members a structured opportunity for reflection, evaluation, and growth. Developed as a tool to help both NMOs and the EHC meet statutory obligations (Article 6.1., adopted in 2024), the OHP also serves to support continuous development — an essential element of a strong, sustainable organisation.

For the EHC community, the OHP was about **empowering NMOs** to become more competent, resilient, independent, and inclusive of all congenital bleeding disorders. It promoted best practices, including democratic and transparent governance, financial accountability, and stable, formalised relationships with stakeholders.

In 2024, the OHP was incorporated into the **Patient Education Fund** (find more information on page 42).

## Youth Fellowship Programme

The Youth Fellowship Programme aims to keep **young people engaged and active within the bleeding disorders community**. In 2024, the EHC continued supporting youth activities by assisting NMOs in organising youth-focused events, and providing support through remote mentoring or in-person visits to these events.

Recognising the value of youth participation, the EHC works to **strengthen connections** among young people and empower their advocacy. The Programme encourages active engagement at both national and international levels, helping young voices shape the future of the bleeding disorders community.

# Events

## Youth Leadership Workshop

5-7 April 2024

The EHC Youth Leadership Workshop has a longstanding tradition dating back to 2014. Over the years, its programme has centred on the core requirements for **successful volunteer engagement** while evolving to meet the current needs and interests of the participants.

In 2024, the Workshop was held from 5 to 7 April in **Brussels**, Belgium, **bringing together 14 participants from 12 different countries**. The event adopted an interactive and participatory approach, fostering active learning through icebreakers, hands-on exercises, case studies, and role-playing activities. It focused on three key modules, offering a series of engaging sessions:

- Fundamentals of **patient organisations** in the context of volunteering
- The importance of adopting and understanding a **strategic process** and collaboration, internal and external **communications**, and medicines development
- Interaction with external stakeholders

Participants also gained valuable insight into the challenges faced by the EHC NMOs across various countries regarding rare bleeding disorders care, based on information collected by the participants before the Workshop.

## Leadership Conference

7-9 June 2024

The 10th edition of the EHC Leadership Conference took place in **Brussels**, Belgium, **welcoming 61 participants from 36 EHC NMOs**, along with expert speakers, EHC staff, and industry representatives.

This annual event serves as a key platform for NMOs to connect, exchange best practices, and discuss emerging challenges in the bleeding disorders community. The conference featured **thematic sessions, panel discussions, and hands-on breakout sessions**, fostering meaningful dialogue on topics such as community growth, new developments in bleeding disorder treatment, and organisational health.

**Programme Highlights**

- **7 June:** The highlight of the day was an Extraordinary General Assembly, during which new Statutes were approved, followed by a community debate and networking activities, including a pleasant 60-minute walk through Brussels' historic Cinquantenaire Park as part of the #ThisWay initiative. This provided a refreshing break for informal conversations and networking while enjoying the fresh air and good weather.
- **8 June:** The programme included clinical updates on bleeding disorder treatments, followed by discussions on perspectives at individual, national, and European levels. Participants engaged in breakout sessions covering key thematic areas such as VWD, Women and Bleeding Disorders, Youth, and Extremely Rare Bleeding Disorders. A poster session showcased national projects, fostering collaboration among NMOs.
- **9 June:** The final day focused on sustainability, relationship-building, and evaluations, introducing a new 'Question Box' for anonymous inquiries addressed by the EHC leadership.

## EHC Annual Conference

4-6 October 2024

The EHC Annual Conference is a **key gathering for the bleeding disorders community**, bringing together patients, HCPs, and industry experts to discuss the latest advancements in treatment, research, and patient care. Hosted in **Sofia**, Bulgaria, by the Bulgarian Haemophilia Association, the 2024 edition welcomed over 300 attendees, including EHC NMO advocates, encouraging knowledge exchange and strengthening community building.

To ensure comprehensive representation, the EHC Conference programme featured expert-led sessions on key topics such as haemophilia in Bulgaria, MDT, VWD, novel therapies, gene therapy, pain management, mental health, ageing, and more. For the first time, each session incorporated both clinicians' and patients' perspectives, enriching discussions with diverse insights.

On **5 October**, the EHC GA took place exclusively for NMO members and patient representatives, featuring live Russian interpretation. During the meeting, the next EHC SC was elected.

On **6 October**, coordinators of the **Patient-Reported Outcomes, Burdens and Experiences (PROBE)** project hosted an open workshop to share the latest insights on patient-reported outcomes. Additionally, the WFH held a dedicated session with its NMOs, in alignment with the EHC-WFH MoU.

## New Technologies Workshop

1-3 November 2024

The New Technologies Workshop plays a vital role in educating EHC NMOs on the latest advancements in novel therapies through engaging presentations and patient-clinician panel discussions. In 2024, the event brought together **70 participants in Helsinki,** Finland, with priority given to one patient representative and one physician per NMO to ensure broad community representation.

The Workshop programme covered replacement and non-replacement therapies, gene therapy, and real-world evidence, along with discussions on novel treatments with the EHC MAG. It also addressed the treatment and care of individuals with extremely rare bleeding disorders, contributing to broader understanding and collaboration across the community.

## ERIN Summit

28 November - 1 December 2024

Building on the success of the EHC Inhibitor Summit, the ERIN Summit has an **8-year tradition of providing a dedicated space for patients with extremely rare bleeding disorders and haemophilia** with inhibitors, along with their families and caregivers. In 2024, the event took place in **Zagreb**, Croatia, bringing together almost 70 attendees.

The Summit programme focused on MDT and support and collaboration between peers.

### Programme Highlights

- **29 November:** Sessions covered specific bleeding disorders — including Glanzmann's thrombasthenia, factor V deficiency, afibrinogenemia, factor VII deficiency, and haemophilia with inhibitors — from both HCP and patient perspectives, followed by peer-to-peer discussions and practical workshops.
- **30 November:** Discussions analysed current and desired treatments, patient-HCP perspectives on care and research development, and continued with peer-to-peer sessions and workshops.
- **1 December:** The final day focused on the role of stakeholders in improving treatment and access, addressing remaining questions, and drawing conclusions from the discussions.

Beyond the insights and knowledge, the **most valuable takeaway** from the ERIN Summit was the **connections formed**. For patients with extremely rare bleeding disorders and haemophilia with inhibitors, as well as their families and caregivers, this event is more than just an educational experience — it's a lifeline. Sharing experiences, exchanging advice, and forming friendships reaffirm that they are not alone.

## Round Tables

### Round Table on Current Issues in VWD

10 September 2024

The EHC hosted its **first post-pandemic Round Table** on Current Issues in VWD at the Warwick Hotel in Brussels, Belgium. The event brought together **20 stakeholders**, including HCPs, patient advocates, and industry representatives, to discuss challenges and advancements in VWD care.

Key discussions covered **diagnosis complexities, the importance of early prophylaxis, and treatment accessibility**. Dr Susie Shapiro highlighted the need for better education and awareness, particularly for women experiencing heavy menstrual bleeding.

**Dr Maria Elisa Mancuso** emphasised the benefits of early intervention, and patient advocate **Julia Rauscher** shared her struggles, underscoring the critical role of self-advocacy and clinician awareness.

The event concluded with a **Q&A session addressing barriers to prophylaxis** and the need for standardised guidelines. The discussions reinforced EHC's commitment to improving VWD care through education, collaboration, and enhanced access to treatment.

The EHC also piloted a travel grant initiative to support NMO participation in this type of event.

Executive Summary

Read the Round Table **Executive Summary**.

### Round Table on Working Together on Rare Diseases – Shared Decision-Making for Advanced Technologies

10 December 2024

The EHC held its second 2024 Round Table on **Working Together on Rare Diseases** – Shared Decision-Making for Advanced Technologies at the Warwick Hotel in Brussels, Belgium, hosting **MEP Vytenis Andriukaitis** (S&D, Lithuania) and **MEP Tomislav Sokol** (EPP, Croatia) as speakers.

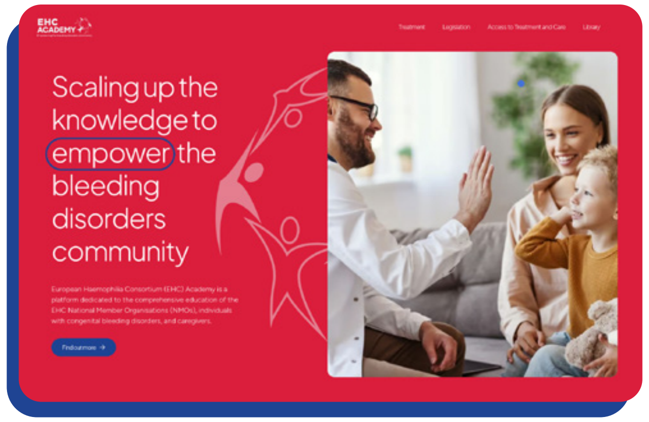
The event brought together **17 participants**, representing a diverse group of stakeholders, including EHC NMO representatives, SC members, and staff. Additional contributors included third-party organisations, clinicians, and industry representatives. This wide-ranging participation enabled a meaningful exchange of perspectives on the key challenges and opportunities in shared decision-making for advanced therapies.

Executive Summary

Read the Round Table **Executive Summary**.

# Projects

## EHC Academy


Scaling up the knowledge to empower the bleeding disorders community

European Haemophilia Consortium (EHC) Academy is a platform dedicated to the comprehensive education of the EHC National Member Organisations (NMOs), individuals with congenital bleeding disorders, and caregivers.

Serving its community has always been at the heart of the EHC's work, and so is patient education. The EHC aims to **ensure access to education for patients and patient organisations** through its diverse educational materials and programmes. To make the experience more inclusive and user-friendly, the EHC has created the EHC Academy, a comprehensive educational platform for its members.

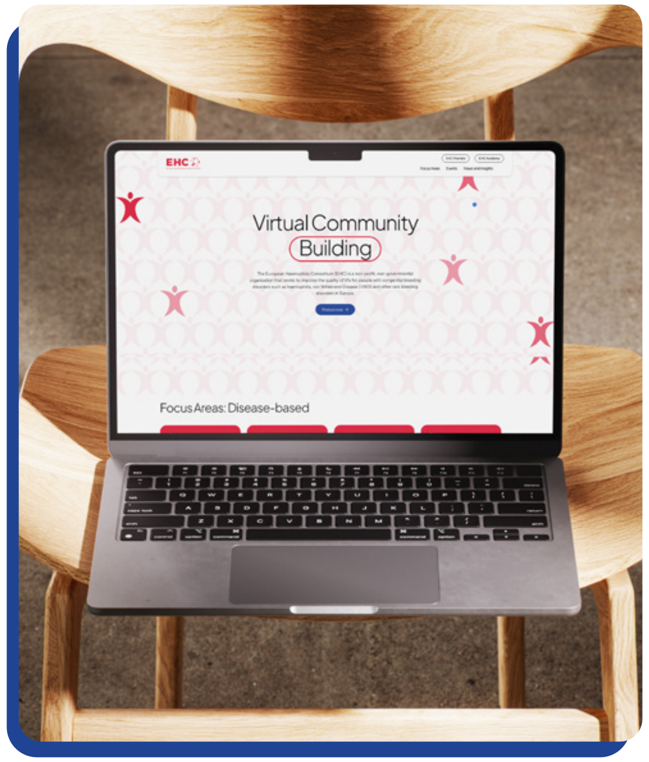
Within the Academy's virtual halls, one can find a wealth of knowledge on bleeding disorders, divided into four main sections: **Treatment, Legislation, Access to Treatment and Care, and Advocacy Materials**. The EHC Academy isn't just about information — it's a dynamic hub of resources. From surveys to care principles, handbooks to guidelines, our platform is a database of patient-centred materials.

Through unique, high-quality knowledge and practical tools, the EHC aims to elevate advocacy skills and pave the way toward a better quality of life for patients across Europe. Our dedication to our mission remains steadfast, encapsulated in our motto: Empowering the Bleeding Disorders Community!

**EHC Academy**

Discover the **EHC Academy**.

## EHC Community Website



The EHC officially launched its Community Website at the EHC Conference in Sofia, Bulgaria in October 2024. This platform brings together the **latest updates from the EHC NMOs, inspiring stories, and insights from the EHC community**.

Dive into the **EHC's work focus areas** – whether aspect-based (such as ageing or women with bleeding disorders) or disease-based (like von Willebrand Disease or extremely rare bleeding disorders). Meet the EHC Committee members, register for the dedicated events, and access carefully selected materials to help navigate the world of bleeding disorders in Europe.

**EHC Community website**

Explore the **EHC Community Website**.

## EHC Website Rebranding: New Look, Same EHC

2024 marked a year of brand refreshment for the EHC. Following the launch of the EHC Academy in April and the EHC Community website in October, the EHC team unveiled its **brand-new main website in December**, completing the transformation of its online platforms.

With updated designs, enhanced features, and seamless integration across all EHC websites, this evolution reflects the organisation's growth. It also provides a more effective way for the community and stakeholders to stay informed about EHC events and activities.

**EHC Website**

Explore the **new EHC Website**.

## EHCucate: A Patient-Centred Educational Resource Evolving with the Treatment Landscape

Since 2021, EHCucate — the EHC's flagship educational project — has been providing trustworthy, impartial, and tailored education for the bleeding disorders community. As the treatment landscape for inherited bleeding disorders rapidly evolves with increasingly complex therapeutic options, EHCucate empowers individuals to navigate these changes confidently. Offering **a personalised learning experience**, it equips patients with the knowledge and confidence needed to actively engage with HCPs and make informed decisions about their care.

### 2024 — A New Chapter for the EHCucate Project

In December 2024, the EHC wrapped up the year with exciting news — EHCucate is now available online as part of the EHC Academy! Over the course of 2024, the project has focused on delivering valuable, up-to-date content to the European bleeding disorders community, while increasing accessibility and inclusivity.

So, what's new?

- A **dedicated EHCucate page** with essential information on bleeding disorders
- A comprehensive **glossary of key terms**
- **Exclusive new content** released on the web platform since December 2024

As the EHC continues transitioning the project online, some topics will remain available in the app version until the process is complete.

**EHCucate online**

Explore **EHCucate online**.

# Patient Education Fund (PEF) and Country Visits

The PEF **evolved from the EHC Think Tank, gradually integrating the OHP, AGs and PAGs** as a multifaceted initiative to enhance knowledge and support for EHC NMOs.

Through country visits, the **PEF provides targeted training and fosters collaboration** with internal and external stakeholders. Additionally, translation and localisation efforts ensure broader accessibility of EHC publications, including NTRs and a Gene Therapy Guidebook. AGs enable NMOs to implement specific programmes that improve the quality of life for people with rare bleeding disorders. Furthermore, webinars covering topics such as rare bleeding disorders, ageing, and the EU legislative landscape offer valuable insights to the community.

In 2024, the PEF allowed, for instance, the translation of the NTR in Hebrew and Arabic, the implementation of many activities in different countries through AGs and the organisation of three EHC Country Visits in Bosnia and Herzegovina, Türkiye, and Russia.

## Country Visit in Bosnia and Herzegovina

**11-12 July 2024**

The EHC embarked on its **first country visit under Miguel Crato's presidency**, joining forces with the WFH to assess the situation for patients with bleeding disorders in Bosnia and Herzegovina and engage with decision-makers on improving care. The packed agenda included **key meetings** with patients, healthcare providers, and officials to **address treatment challenges and advocate for better access to care**, especially beyond Sarajevo. Discussions at the Fund Institute focused on updating drug lists, expanding patient representation in procurement, and improving nationwide care. A highlight was the **visit to the Sarajevo Hemophilia Treatment and Comprehensive Care Centre**, where the delegation met the MDT, toured facilities, and discussed the upcoming EAHAD Comprehensive Care Centre Certification renewal.

The EHC thanks its Bosnian and Herzegovinian NMO, **Udruženje Hemofiličara Bosne i Hercegovine**, for their warm welcome and remains committed to improving care and quality of life for all patients.

## Country Visit in Türkiye

**7 October 2024**

The delegation visited the **Istanbul University Hemophilia Care Center**, where they discussed the situation regarding bleeding disorder patients, diagnostics, and access to treatment and care in Türkiye with Dr Başak Koç Şenol (EHC VWD Committee Member). The EHC also had a chance to meet patients and talk to the study coordinator about clinical trials in the country.
At the Department of Orthopedics and Traumatology, Prof Dr Gökhan Polat shared that two operations are performed daily with one surgery per week being for haemophilia patients. Training of the physiotherapy students also takes place at the Department.

On the same day, the EHC delegation met with Prof Dr Bülent Zülfikar, Rector of the Istanbul University and President of Türkiye Hemofili Derneği (the EHC Turkish NMO), to discuss relations between the NMO and the EHC.

Positive expectations from the EHC Conference in 2026 in Istanbul were expressed.

In addition, the EHC visited the **Hemophilia Society of Türkiye** to meet with patients and board members. The future EHC Conference, youth development, and other opportunities for the NMO members at the EHC were discussed, among others.

The meeting took place in the NMO's headquarters, so the EHC delegation could also assess the treatment room, playroom for kids, conference rooms, psychologist cabinet, and impressive physiotherapy facility.

## Country Visit in Russia

**9-12 October 2024**

During the four-day visit, the EHC delegation had an opportunity to not only **meet both patients and the board** of **Всероссийское общество гемофилии**, the EHC Russian NMO, but also to **engage with clinicians and support the advocacy efforts** of the NMO at the national level.

The EHC paid a visit to the **HTC and the Department of Traumatology and Reconstructive Orthopedics** for Patients with Hemophilia to meet with staff and patients, and assess the situation regarding bleeding disorders care in Russia. On 12 October, the **10th All-Russian Scientific and Practical Conference** on Hemophilia, titled *Issues of Organisation, Diagnosis, and Treatment of Patients with Hereditary Coagulopathies* took place, where the discussion around treatment and care continued on the stage.

The EHC delegation also attended the **board meeting of the All-Russian Haemophilia Society** and a **regional meeting of seven EHC NMOs** (Armenia, Belarus, Georgia, Kyrgyzstan, Russia, Tajikistan, Uzbekistan) together with Kazakhstan and Turkmenistan. All NMOs had an opportunity to present the current state of bleeding disorder treatment and care in their respective countries, and express the needs for which they seek WFH and EHC help and support.

The Russian NMO reported a positive outcome following the EHC visit, with the Ministry of Health initiating the assessment process to address improvements in care for patients with hereditary coagulopathies. Progress was also made in the approval process for clinical recommendations for VWD patients. Another positive outcome was the inclusion of factor concentrates in the state-covered cost of high-tech medical care in orthopaedy for patients with haemophilia. Additionally, the overall budget for such surgeries was increased.

08

# 2024 Financial Reporting

Aiming to demonstrate the EHC's organisational health, and ensure its continued growth and sustainability.

## Independent Auditor's Review Report

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**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 2024, and the statement of comprehensive income, statement of changes in equity and, and a summary of significant accounting policies and other explanatory information. The balance sheet shows a total of € 2.491.173,73 and a profit for the year of € 3.416,74.

**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.

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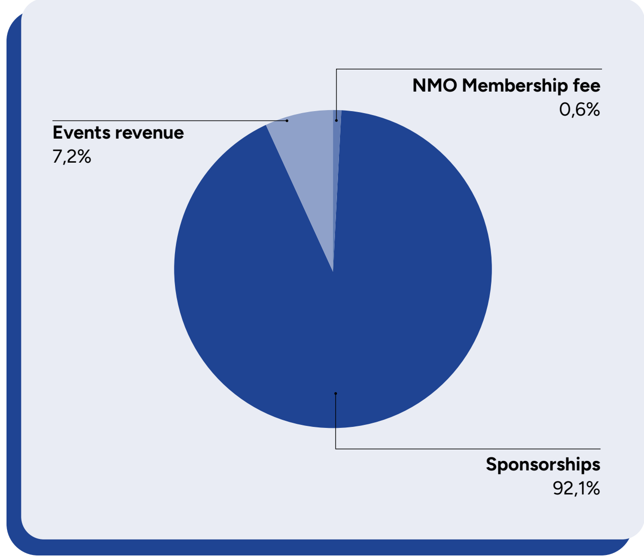
**EHC Audit Report 2024**

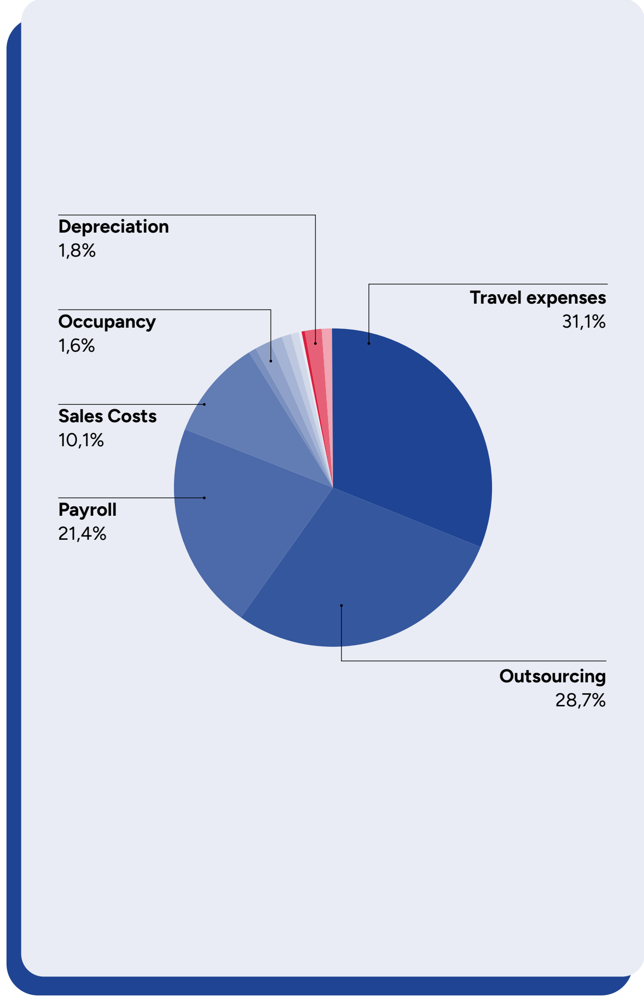
Download the **EHC Audit Report 2024**

# EHC Income



| Income | Finals (€) |
|---|---|
| NMO Membership Fee | 11.100,00 |
| Sponsorships | 1.674.000,00 |
| Event(s) revenue | 131.144,15 |
| Other Operating Income | 1.380,47 |
| Discount, allowances and rebates | -30.000,00 |
| **Gross income** | **1.787.442,06** |
| Volunteers' Contribution | 265.800 |

# EHC Expenditure



| Expenditure | Finals (€) |
|---|---|
| Travel Expenses | 554.202,33 |
| Outsourcing | 511.162,99 |
| Payroll Costs | 369.929,22 |
| Sales Costs | 179.478,97 |
| Occupancy | 28.834,36 |
| Office Costs | 22.065,59 |
| Other personnel costs | 18.205,97 |
| Services | 17.059,72 |
| General Costs | 8.359,42 |
| Insurances | 4.122,71 |
| Marketing | 4.788,49 |
| Depreciation | 48.264,01 |
| Other operating costs and financial result | 16.524,78 |
| Income tax on result | 1.026,76 |
| **Total expenditure** | **1.784.025,32** |

# EHC Balance Sheet 2024 as of December 2024

## Assets

| Fixed assets | Amount | % |
|---|---|---|
| Intangible fixed assets (research and development) | 30.557,23 | 1.2% |
| Tangible fixed assets (buildings, lands, machinery, equipment, furniture and vehicles) | 406.339,78 | 15.6% |
| Financial fixed assets (cash guarantees) | 87,80 | 0.003% |
| **Total fixed assets** | **436.984,81** | **16.9%** |

| Current assets | Amount | % |
|---|---|---|
| Amounts receivable within one year | 85.037,68 | 2.7% |
| Transitory assets | 7.626,15 | 7.06% |
| Cash and cash equivalents | 1.961.525,09 | 73.2% |
| **Total current assets** | **2.054.188,92** | **83.07%** |
| **Total assets** | **2.491.173,73** | **100%** |

## Liabilities

| Liabilities | Amount | % |
|---|---|---|
| Equity | 2.227.584,79 | 83.06% |
| Debts | 263.588,94 | 16.93% |
| **Total liabilities** | **2.491.173,73** | **100%** |

## 09

# Corporate Giving Partners

The EHC wishes to acknowledge and thank its **2024 Corporate Giving Partners** for making its work possible.

## Platinum sponsors

**BioMarin**

**Novo Nordisk**

**Roche**

**Sanofi**

**Sobi**

## Gold sponsors

**CSL Behring**

**Regeneron**

## Silver sponsors

**Kedrion**

**LFB**

**Takeda**

The EHC also wishes to thank the following companies for **supporting individual 2024 projects and events**:

- EHC Conference was supported by BioMarin, CSL Behring, Novo Nordisk, Pfizer, Roche, Sanofi, and Sobi
- EHCucate project was supported by Roche and Sobi
- ERIN was supported by Kedrion, Roche and Sanofi
- Health Economics Course was supported by Pfizer and Sobi
- Leadership Conference was supported by Sanofi and Sobi
- New Technology Workshop was supported by BioMarin, Regeneron, Sanofi, and Sobi
- Patient Education Fund was supported by Biotest, Roche, Sobi and Takeda
- Round Tables were supported by BioMarin, CSL Behring, Regeneron, and Sobi
- Think Tank was supported by CSL Behring, Novo Nordisk, and Sobi
- #ThisWay Campaign was supported by LFB and Sobi
- VWD community's work was supported by CSL Behring and Takeda
- Youth Fellowship Programme was supported by Sobi
- Youth Leadership Workshop was supported by Sanofi and Sobi

## 10

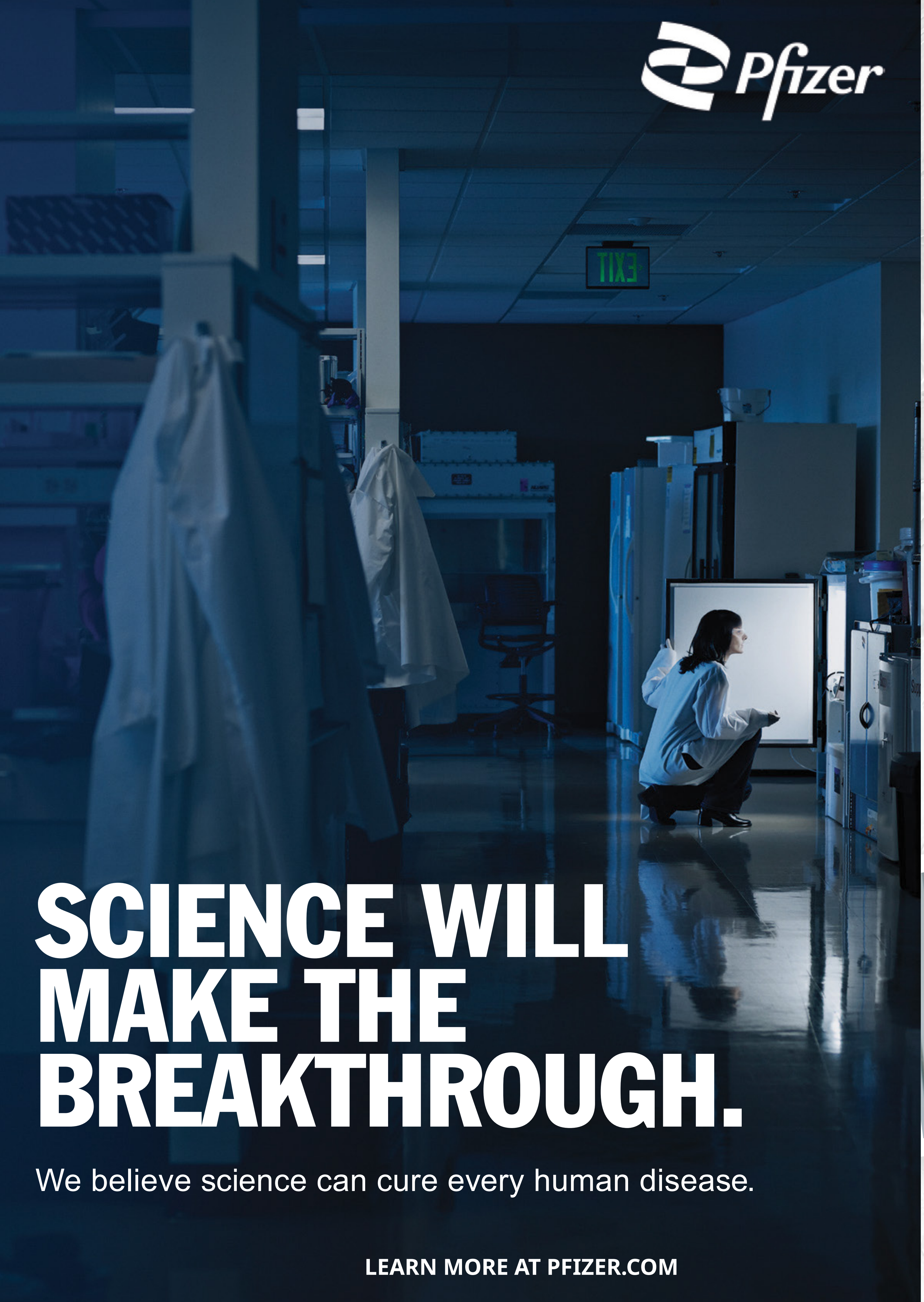
# Volunteers

The work of the EHC would **not be possible without the support of dozens of volunteers from around Europe** who generously offer their time and energy to make the EHC's activities possible.
In 2024, the work of the EHC was supported by a total of 77 volunteers, who all together dedicated 4.430 hours of their time to the EHC.

| 77 | 4.430 |
|---|---|
| **Total volunteers** | **Volunteer hours** |

The EHC is immensely grateful to all of you for your energy and dedication!

## Thank you!



Beyond the Bleed

Sanofi is going ***Beyond the Bleed*** to meaningfully advance the care paradigm and raise awareness of the impact of hemophilia through the eyes of people living with it.

Hemophilia is more than just bleeding episodes — it affects relationships, health, career, travel and more. By addressing these challenges together, our ambition is that people living with hemophilia, their families, and caregivers can live confidently in the present and hopeful for the future.

*To learn more, visit our website or scan the QR code*

sanofi

*Photos are real people with hemophilia

MAT-GLB-2500854 v1.0 03/2025

# Strategic Patient Community Collaboration Areas until 2030

Roche

Recognising the TREATMENT BURDEN of different patient subpopulations and enabling patients to CHOOSE the best therapy offering, striving towards normalisation

Advancing the ADVOCACY impact of patient communities and creating broad ACCESS to novel therapies for more people that need prophylaxis based on their phenotype

UNDERSTANDING the NEEDS of underserved parts of the communities, enabling actionable insights to shape our development strategies, enable patient access, and improve outcomes

Find out more at

Veeva M-XX-00019754, February 2025

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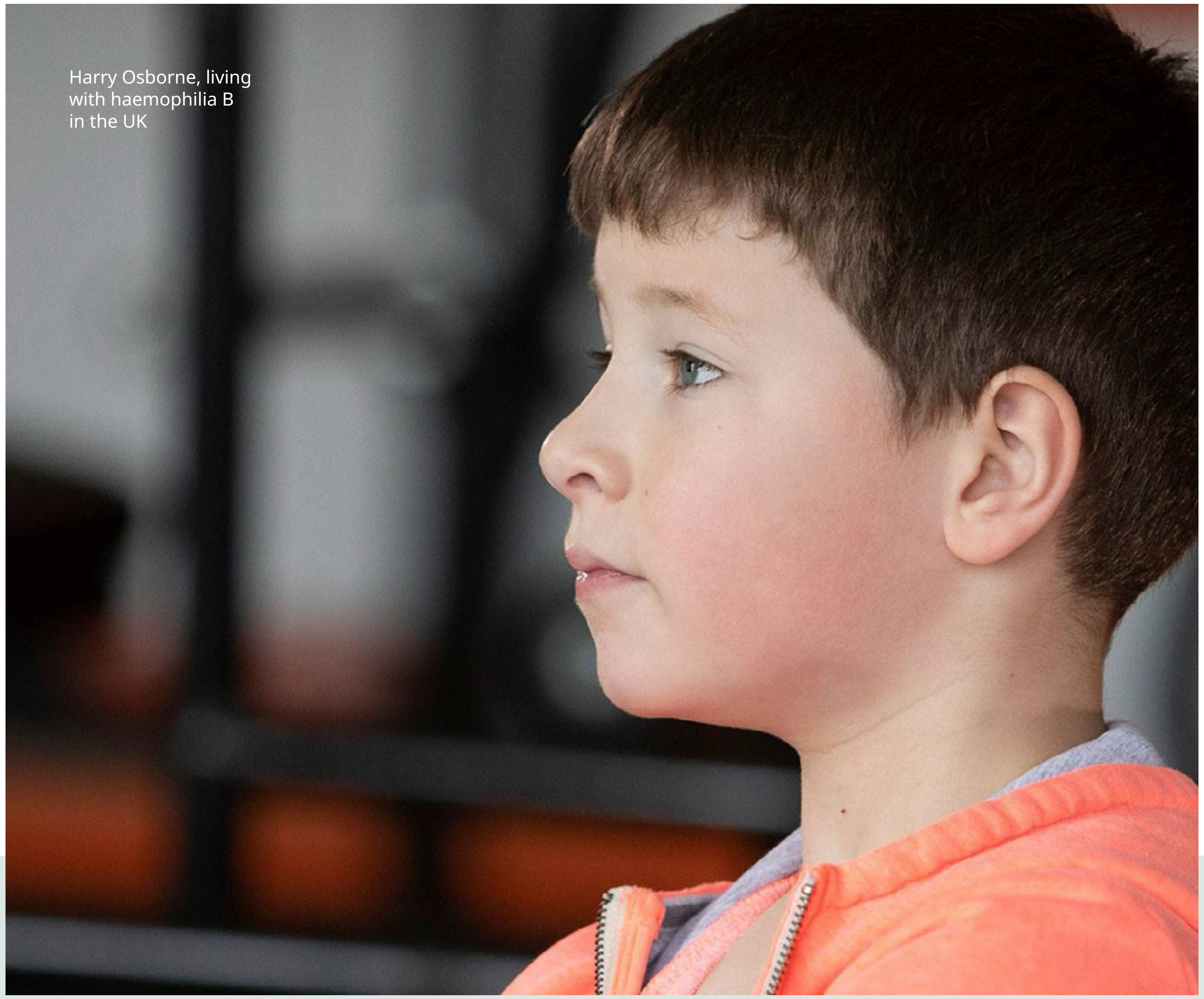

Harry Osborne, living with haemophilia B in the UK

## **Proud to partner with the EHC** to empower the community for better outcomes

**Driving change** | in rare disease

novo nordisk®

# Learn differently.
# Be involved differently.
# HaemDifferently.

**Haem**differently
Presented by BIOMARIN

HaemDifferently would like to have an open and transparent exchange with you about the subject of gene therapy research.

Contact us or find out more at:
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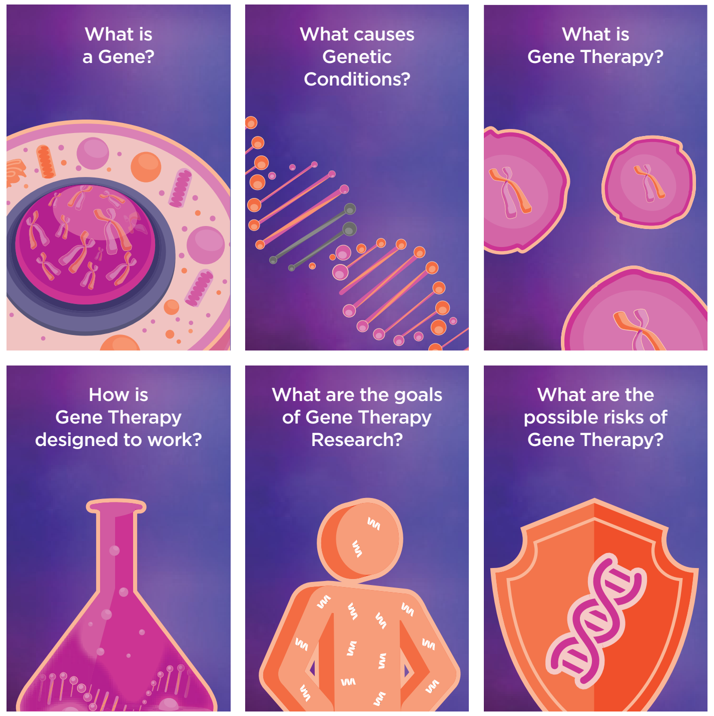

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Developed and funded by BioMarin | May 2024

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**CSL Behring**

**Yesterday, today and in the future -**
**Living better with bleeding disorders**

EUR-OTH-0291 05/2024

**CSL Behring, driven by our promise to deliver innovation to people with bleeding disorders**

### Tradition and progress are in our DNA!

For over 50 years, we contributed significantly to major advancement in the treatment of bleeding disorders, such as haemophilia A / B and von Willebrand disease, with many of them being the first of their kind. Now and in the future we will continue to develop innovative and proven treatments that will improve the lives of people with bleeding disorders.

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LIBERATE
**LIFE**

# REIMAGINE YOUR STORY

## Taking one step forward in living life beyond haemophilia

In July 2024, Sobi supported a group of people living with haemophilia to climb Mont Blanc, the highest peak in the Alps. The expedition saw the climbers attempt to scale the mountain, inspiring others with haemophilia to embark on a journey to identify and overcome their own personal challenges and reimagine the possibilities of life with haemophilia.

We hope their story shows that, with the right support, it is possible to take steps towards personal goals, no matter how big or small, and potentially live a fuller life with haemophilia. Whether it's going to a concert, joining a social activity, dancing, or practicing a new sport, working with your care team can potentially support you in exploring a better life with haemophilia.

**What is your personal 'Mont Blanc' challenge?**

sobi
rare strength

**Discover more about their incredible journey and find inspiration for your own personal goals by watching the film**

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Date of preparation: March 2025, NP-40243

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