EHC

european haemophilia consortium

Advocating for people with haemophilia and congenital bleeding disorders

ANNUAL REPORT
2023

Miguel Crato
EHC President

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

Message from the EHC President

In 2023, the European Haemophilia Consortium (EHC) experienced significant transitions in its leadership and staff, marking a year of change and adjustment. With new faces in key positions, the organisation needed to reassess its objectives and mission to align with the fresh perspectives and goals brought by the new team. The period of transformation necessitated a comprehensive review of existing strategies and the implementation of new approaches to ensure continuity and effectiveness in achieving the EHC's overarching mission.

Amid these changes, the EHC recognised the importance of shifting its focus towards its National Member Organisations (NMOs). The evolving landscape highlighted the need for stronger support and collaboration with our members to better address their specific challenges and make the most out of the opportunities. By prioritising NMOs, the EHC aimed to foster a more cohesive and responsive network, enhancing the overall impact of its initiatives. This strategic pivot was essential to maintaining the organisation's relevance and effectiveness during a time of internal reorganisation and external demands.

In 2023, we continued to keep our community connected, educated, and empowered through various projects, events, and initiatives. From the EHC Leadership Conference in Brussels in June with a focus on the Principles of Good Organisational Health to the success of the EHC Conference in Zagreb, Croatia, in October which hit the record in the number of attendees.

In November, we met in Portugal for the New Technologies Workshop which traditionally provided the community with the latest updates in the treatment landscape. 2023 also saw a big change in the approach towards education - our Health Economics Workshop moved online and from 2023 onwards is held in the form of a course enhancing inclusion and accessibility.

Rethinking and restrengthening – the EHC Committees worked hard to move forward with their agenda last year. The success of the Youth Fellowship Programme helped us to continue to support many of our international participants. Youth Leadership Workshop – a flagship event of the Youth Committee – together with traditional Youth debates at the EHC Conference and other local and European projects ensured that the spirit of the Youth Leadership Alumni remains engaged and active within the community.

On February 1, 2023, the EHC Von Willebrand Disease (VWD) Committee marked the first-ever European VWD Awareness Day with a bruise campaign How Did That Happen launched. The campaign which got viral also outside of the EHC paved the way for the future VWD identity. The Let's Talk VWD series, together with the community engagement initiatives, also created the foundation for the big projects launched later in 2024.

Building upon the success of the Inhibitor Working Group (IWG), in 2023 the new European Rare and Inhibitor Network (ERIN) Committee continued its work on the development of the programmes and initiatives, now including extremely rare bleeding disorders, and advocating for overcoming the challenges the extremely rare bleeding community faces in their everyday life.

The EHC Women and Bleeding Disorders (WBD) Committee's key activities in 2023 focused on diagnosis, care pathways, engaging women, and the importance of mental health and self-care. The Committee shared podcasts and marked International Women's Day with a campaign to raise awareness and demand better access to treatment, care, and research for women and girls.

Importantly, in 2023, the EHC improved the effectiveness of dialogue: fostering deeper internal conversations within our own communities to better support a wider range of bleeding disorder groups; and redesigning collaboration externally. The new Memorandum of Understanding (MoU) between the EHC and the World Federation of Hemophilia (WFH) is signed. Our continued dialogue with the European Association for Haemophilia and Allied Disorders (EAHAD) and communication with the European Union (EU) institutions about the unmet health and medical needs of our community, including collaboration with the ERN-EUroBloodNet, had some progress in 2023.

The EHC recognises the critical need to reinvigorate even more our collaboration with stakeholders and enhance our visibility at the European level. Strengthening these relationships is essential for advancing our mission and effectively advocating for the needs of our community. By fostering closer partnerships and actively engaging with European institutions, we can ensure that our collective voice is heard and that our efforts have a greater impact. We must work together to position the EHC as a prominent and influential advocate, driving positive change, and shaping policies that benefit all our members.

We extend our heartfelt gratitude to important people who left the EHC in 2023: Amanda Bok, our former Chief Executive Officer (CEO), Declan Noone, our former President, and Manon Degenaar-Dujardin, our former Vice President of Finance, for their invaluable contributions to the EHC. Their dedication, vision, and leadership have been instrumental in shaping the organisation and driving its mission forward. Amanda's strategic guidance, Declan's inspiring leadership, and Manon's meticulous financial stewardship have left a lasting legacy that will continue to contribute to the EHC for years to come. We wish them all the best in their future endeavours and thank them sincerely for their unwavering commitment and service.

We urge our community to become more actively involved with the EHC, as the strength and success of our organisation are intrinsically tied to the engagement and support of our NMOs. The EHC is not just an entity: the EHC is a collective representation of all NMOs and, together, we can drive meaningful change and advocate more effectively for our shared goals.

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>
> **Strengthening these relationships is essential for advancing our mission and effectively advocating for the needs of our community.**


00:14:56
REFLECT, PLAN, ACT
Dealing with mental health with a chronic condition with focus on Central and Eastern Europe

EHC
european haemophilia consortium

Advocating for people with haemophilia and congenital bleeding disorders

ABOUT US

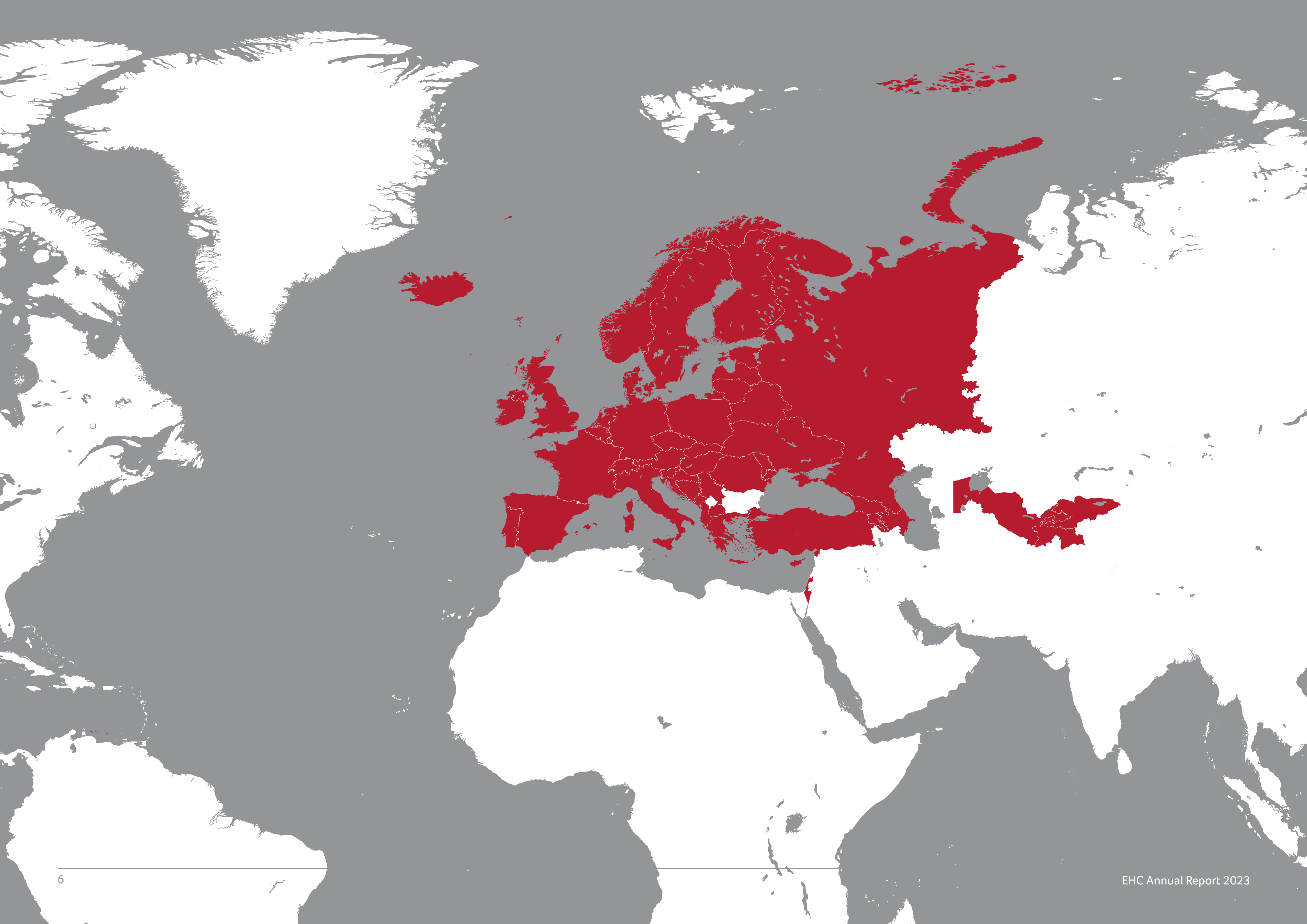
The EHC is an international non-profit organisation representing 48 national patients' organisations for people with rare bleeding disorders from 27 Member States of the EU and most Member States of the Council of Europe.

The EHC represents more than 120,000 people diagnosed with rare bleeding conditions such as haemophilia, 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 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, 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.



Membership

In 2023, the following organisations 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 Herzegovina	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	עלה – עמותה לחולי המופיליה
Italy	Federazione delle Associazioni Emofilici (Fedemo)
Kyrgyz Republic	Общественное объединение больных гемофилией Кыргызской Республики
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	Общественная организация «Гемофилия»
Turkey	Türkiye Hemofili Derneği
Ukraine	Всеукраїнське товариство Гемофілії
United Kingdom	The Haemophilia Society
Uzbekistan	Узбекское общество гемофилии

Steering Committee

By July 2023, the EHC Steering Committee (SC) said goodbye to Declan Noone, who resigned from his role as President. Elected in 2019, Noone focused on providing members with accurate scientific and medical information, as well as continued data-based advocacy focusing especially on underserved patient populations such as women and people with von Willebrand Disease and extremely rare bleeding disorders.

To ensure continuity and progress, the EHC SC unanimously appointed Olivia Romero Lux to assume the role of President in an interim caretaking capacity until the end of the term in October 2023. With the regular election held during the EHC 2023 General Assembly, then, Miguel Crato was elected as the latest EHC President.

Manon Degenaar-Dujardin continued to fulfill her duties as Vice President of Finance until November 2023, when she resigned. The EHC SC appointed its committed member Jo Eerens as her successor for the remaining two years of the mandate until the next elections in October 2024.

In November 2023, Admir Mehić from Bosnia and Herzegovina was co-opted as SC member, followed in December by Panagiotis Christoforou from Greece.

The EHC 2023 Steering Committee Members were:

Declan Noone
President
(Until July 2023)
Irish NMO

Manon Degenaar-Dujardin
Vice President of Finance
(Until November 2023)
Dutch NMO

Olivia Romero Lux
Co-opted SC Member
Interim President
(July - Oct 2023)
French NMO

Miguel Crato
SC Member
(Until October 2023)
President
(Since October 2023)
Portuguese NMO

Jo Eerens
SC Member
(Until October 2023)
Vice President of Finance
(Since November 2023)
Belgian NMO

Amanda Bok
Ex-officio Member
CEO
(Until February 2023)

Luis Teixeira
Ex-officio Member
CEO
(Since May 2023)

Evelyn Grimberg
Co-opted SC Member
Dutch NMO

Diana Lighezan
Co-opted SC Member
Romanian NMO

Stefan Radovanovic
Co-opted SC Member
Serbian NMO

Admir Mehić
Co-opted SC Member
Bosnian and Herzegovinian NMO
(Since November 2023)

Panagiotis Christoforou
Co-opted SC Member
Greek NMO
(Since December 2023)

EHC Medical Advisory Group

The EHC Medical Advisory Group (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 2023, the MAG members were:

- **Prof Jan Blatny** | Czech Republic
- **Prof Ana Boban** | Croatia (Since October 2023)
- **Prof Michael Makris (Chair)** | United Kingdom (Until September 2023)
- **Dr Maria Elisa Mancuso (Chair)** | Italy (Since October 2023)
- **Prof Johannes Oldenburg** | Germany
- **Prof Flora Peyvandi** | Italy (Until September 2023)

Following the resignation of Prof Michael Makris and Prof Flora Peyvandi in September 2023, Prof Ana Boban and Dr Maria Elisa Mancuso joined the EHC MAG. Dr Maria Elisa Mancuso was elected as Chair of the Group.

EHC Medical and Scientific Advisory Group (MASAG)

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

In 2023, the MASAG members 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
- **Radoslaw Kaczmarek** | USA
- **Evelien Mauser-Bunschoten** | The Netherlands
- **Beatrice Nolan** | Ireland
- **Cristina Novembrino** | Italy
- **Jamie O’Hara** | United Kingdom
- **Brian O’Mahony** | Ireland
- **Debra Pollard** | United Kingdom
- **Frits Rosendaal** | The Netherlands
- **Luigi Solimeno** | Italy
- **Thierry Vanden Driessche** | Belgium

EHC Staff & Organisation

As of January 2023, the EHC staff entered a period of some major changes. In February, after a decade of dedicated work, Amand Bok resigned from her position as CEO. In her decade of work, she had an enormous impact on the EHC as an organisation and the European bleeding disorders community.

In May 2023, then, Luis Teixeira was hired as the new CEO, bringing a wealth of experience from different European associations.

By the end of 2023, however, other dedicated members of the staff left their positions: Jeanette Fava as Operations Director – replaced for six months by Shinoh Lee; Saskia Pfeyffer as Office Assistant; and Naja Skouw-Rasmussen as Innovation Lead.

Amanda Bok
CEO
(Until March 2023)

Luis Teixeira
CEO
(Since May 2023)

Fiona Brennan
Patient Engagement Lead

Daria Camilli
Communications and Education Lead

Jeanette Fava

Operations Director
(Until March 2023)

Zita Gacser

Behavioural Change Lead

Kristine Jansone

Community Development Lead

Shinoh Lee

Operations Director
(March to September 2023)

Rumana Molla

Administration Officer

Saskia Pfeyffer

Office Assistant
(Until October 2023)

Laura Savini

Advocacy Lead

Naja Skouw-Rasmussen

Innovation Lead
(Until December 2023)

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 partnerships and advocacy: in substance, in form, and into the future.

The EHC's day-to-day work is guided by its Strategic Objectives, developed every four years by the EHC SC, MAG, MASAG and Staff, based on the needs of the bleeding disorders community.

In 2023, the EHC Strategic Objectives 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 2023
- World Haemophilia Week 2023
- EHC Conference 2023
- EHC Think Tank
- 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 New Technologies Workshop
- EHC Health Economics Course
- Educational App EHCucate
- Novel Treatment Review
- Public Policy Reviews

3

Community Empowerment and Support

To empower NMOs and support their future development.

- #ThisWay Campaign
- EHC Youth Fellowship Programme
- EHC Leadership Conference 2023
- Women and Bleeding Disorders
- Von Willebrand Disease
- European Rare and Inhibitor Network

4

Sustainability

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

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

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 2023

28 FEBRUARY 2023

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

Since its creation in 2008, Rare Disease Day has played a critical part in building an international rare disease community that is multi-disease, global, and diverse – but united in purpose. Observed every year on February 28th (or 29th in leap years) – the rarest day of the year – Rare Disease Day was set up and is coordinated by EURORDIS and 65+ national alliance patient organisation partners.

To the EHC community, Rare Disease Day means bringing attention to those rare among rare, broadening focus and including in the conversation those who have not always been included, those who are still struggling to receive accurate or any diagnosis at all.

To mark Rare Disease Day on 28 February 2023, the EHC European Rare and Inhibitor Network (ERIN) Committee launched a brief but comprehensive campaign on social media highlighting the key points of the ERIN Manifesto co-created and signed by participants of the ERIN Summit in December 2022. The ERIN Manifesto outlines the needs of patients with extremely rare bleeding disorders, the challenges they face in having these needs met, and the support that they would require to have these needs met at the European and national levels.

The goal of the EHC Rare Disease Day 2023 social media campaign was to call for persons affected by extremely rare bleeding disorders or haemophilia with inhibitors to share their most urgent unmet need via an online survey to further integrate the ERIN Manifesto.


ERIN

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.

People Affected by
Extremely Rare Bleeding Disorders


ERIN

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.

People Affected by
Haemophilia B with Inhibitors


ERIN

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.

People Affected by
Haemophilia A with Inhibitors

ADVOCACY AND AWARENESS

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World Haemophilia Week 2023

17-21 APRIL 2023

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

In April 2023, around WHD, the EHC organised the 1-week awareness campaign *On a Pathway Towards Healthy Ageing*, a theme that is equally important for all groups of the bleeding disorders community. During the week from Monday 17th to Friday 21st, the EHC addressed different aspects of this theme.

The EHC is deeply grateful to all the volunteers and healthcare professionals who contributed to the content creation and planning of the World Haemophilia Week 2023.

April 17

On its main communication channels, the EHC published an address by the EHC President Declan Noone and a detailed infographic outlining the outcomes from the EHC Needs Assessment Survey of bleeding disorder patients aged 50+.

www.youtube.com/watch?v=Kkqbz1A6ano

April 18

The EHC focused on the importance of access to multidisciplinary care as the life expectancy of people living with bleeding disorders has reached that of the general population. To set the scene, the EHC released the video of its webinar On a Pathway Towards Healthy Ageing: Importance of Multidisciplinary Care and Coordination. Speakers were Dr Kate Khair and Prof Cedric Hermans discussing challenges and considerations on multidisciplinary care from the perspectives of nursing care and haematology. The EHC also selected a few relevant materials from its series Happy ½ Hour with an Expert in Haemophilia Care, which focuses on multidisciplinary care for people with inhibitors.

www.youtube.com/watch?v=rOE2OLMomG0&t=2s

April 19

On World Liver Day, the EHC held a webinar with prominent experts on the topic Liver health: focus on care post-viral infection and post gene therapy. The webinar featured Declan Noone (EHC President, moderator), Brian O'Mahony (Ireland), Clive Smith (United Kingdom), and Prof Vincenzo La Mura (Italy). This event had also interpretation in Russian.

April 20

The EHC held a peer–to–peer day with an online discussion with the bleeding disorders community on What does healthy ageing mean for you?, creating a virtual gallery collecting different perspectives.

www.youtube.com/watch?v=8g86TKzS2pw

EHC Conference 2023

EHC Conference 2023

6-8 OCTOBER 2023

The annual EHC Conference is 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, and avail of the opportunity for informal exchange and community building.

Taking place from 6 to 8 October in Zagreb, Croatia, the 2023 EHC Conference brought together 400 attendees including patient advocates representing EHC NMOs, healthcare professionals, and industry experts, to share the latest advancements in treatment, research findings, and best practices in patient care throughout Europe.

The programme of the conference, packed with inspiring talks from a variety of excellent experts, focused on the following topics:

- Haemophilia in Croatia,
- Multidisciplinary Care,
- Von Willebrand Disease,
- European Rare and Inhibitor Network,
- Women and Bleeding Disorders,
- Novel Therapies,
- Pain Management,
- Gene Therapy,
- Mental Health,
- Youth Debate,
- SLAMS,
- Ageing and Bleeding Disorders.

The EHC 2023 Conference was an entirely in-person meeting. Understanding that not all EHC NMOs were able to attend, the EHC considered it paramount to share with them a written summary of selected highlights from four sessions, selected for their significant strategic importance in the evolving bleeding disorders landscape. These four sessions focused on Mental Health, Ageing, Von Willebrand Disease, and Extremely Rare Bleeding Disorders (EHC ERIN Programme).

The EHC 2023 General Assembly (GA) also took place on October 6th, late afternoon. It was accessible to NMOs members and patient representatives only, in English with live Russian interpretation.

On this occasion, Miguel Crato was elected as new EHC President to succeed Olivia Romero Lux – who was nominated Interim President when Declan Noon resigned in summer 2023. Several changes in the MAG were also sanctioned during the GA: the EHC had to say goodbye to a few outstanding members, Prof Michael Makris and Prof Flora Peyvandi; while welcoming new members such as Prof Ana Boban and Dr Maria Elisa Mancuso.

Several side events took place throughout the weekend, like EHC sponsors' symposia – organised by Novo Nordisk, Sobi, Roche, Sanofi, BioMarin, CSL Behring.

Held for the second time in the Conference's history, the EHC Volunteers Mixer on October 5th was again a success. This satellite event brought together the volunteers of the many EHC committees and working groups, serving as an opportunity to exchange best practices. The goal is to strengthen the sense of belonging to the bleeding disorders community.

On October 7th, the World Federation of Hemophilia (WFH) invited its members to join a breakfast meeting at the Conference hotel in Zagreb.

On October 8th, the Patient-Reported Outcomes, Burdens and Experiences (PROBE) project coordinators organised a workshop open to all Conference participants to present the latest updates on the project.

Quelle heure est-il au paradis?
Anne
Nicholas
Elise
Laurence
Lutz

EHC THINK TANK

In 2023, the EHC Think Tank had a bustling year, managing four ongoing workstreams and organising its inaugural face-to-face workshop – the so-called Innovation Summit in Brussels, Belgium. Throughout the year, we conducted a total of 18 virtual workshops dedicated to the Registries, Patient Agency, Access Equity, and Future Care Pathways workstreams.

The Access Equity and Future Care Pathways workstreams began their efforts in early 2023. Workstream members collaborated extensively, actively seeking to understand the root causes behind the current challenges in their specific thematic areas. In June 2023 the EHC hosted members from the Registries and Patient Agency workstreams at the Innovation Summit in Brussels. Employing design thinking approaches, each workstream developed its selected concepts with the guidance of seasoned facilitators and external advisors.

You can find more information on the EHC Think Tank website: **ehcthinktank.eu/about-the-workstreams**

The EHC wants to express sincere gratitude to the workstream members for their invaluable contributions. Heartfelt thanks also to the Think Tank Council and Faculty for their exceptional guidance in shaping the thematic focus and employing methodologies.

OUTCOMES

- Access Equity System Map **https://embed.kumu.io/2cf09bb33720ab7c3f424af8aaff46c5#access-equity-map-full**
- Future Care Pathways System Map **https://embed.kumu.io/57bffb8ec007328773305c91be6904c1#future-care-pathways**

PUBLICATIONS

- Bok A, Noone D, Skouw-Rasmussen N. Short- and longer-term goals for change – A report from the 2nd workshops of the EHC Think Tank Workstreams on Registries, the Hub and Spoke Model and Patient Agency. The Journal of Haemophilia Practice 2023; 10(1): 1-10. **https://sciendo.com/article/10.2478/jhp-2023-0001**
- Skouw-Rasmussen N, Savini L, EHC Think Tank. Access equity: key questions and challenges – A report from the 1st workshop of the European Haemophilia Consortium (EHC) Think Tank Workstream on Access Equity. J Haem Pract 2023; 10(1): 56-61. **https://sciendo.com/article/10.2478/jhp-2023-0011**
- Skouw-Rasmussen N, Savini L. Future Care Pathways – A report from the 1st workshop of the EHC Think Tank Workstream on Future Care Pathways. The Journal of Haemophilia Practice 2023; 10(1): 74-81. **https://sciendo.com/article/10.2478/jhp-2023-0013**
- Gacser Z, Skouw-Rasmussen N, et al. Short- and longer-term goals for change – A report from the 2nd workshop of the EHC Think Tank Workstreams on Access Equity and Future Care Pathways. The Journal of Haemophilia Practice 2023; 10(1): 155-163. **https://sciendo.com/article/10.2478/jhp-2023-0023**

ADVOCACY AND AWARENESS

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

Workstreams

REGISTRIES

The Registries workstream aims to better align registries on bleeding disorders across Europe and beyond. It is perceived that alignment will foster greater opportunities for data sharing, improve data quality, and optimise data collection, to mention a few.

Ultimately, more alignment of registries and data should enable us to answer scientific questions with more data and thereby evolve our understanding of the diseases that can lead to better treatment and care.

ehcthinktank.eu/workstream/workstream-one-registries

PATIENT AGENCY

Patient agency refers to the patient's ability to act. It is a concept that encompasses all levels of patient engagement in light of people's preferences, behaviours, and mindsets.

The workstream has dived into diverse aspects where the patient agency is relevant, such as more equal collaboration between stakeholders, patient experience data or real-world data, and training and education of stakeholders in the healthcare system to have a broader understanding of people who are also patients.

ehcthinktank.eu/workstream/workstream-three-patient-agency

ACCESS EQUITY

In Access Equity, 'access' refers to the ability to benefit from a thing, either financial, physical, etc., while 'equity' refers to the quality of being fair and impartial across and within countries as well as on community and individual levels.

This workstream has identified narrative and behaviour as two key aspects of why access equity is lacking and is currently exploring what it will take to change the narratives and behaviours leading to fairer access to treatment and care.

ehcthinktank.eu/workstream/workstream-four-access-equity

FUTURE CARE PATHWAYS

The Future Care Pathways workstream is keen to uncover which perspectives are crucial to consider when working on care pathways. At the centre of their exploration are:

- How to combine digital and human elements in one pathway;
- How problematic behaviours, nudges, and incentives prevent the care pathway from improving;
- The importance of data-driven pathways to document the current process and facilitate improvements of care pathways.

ehcthinktank.eu/workstream/workstream-five-future-care-pathways

HUB & SPOKE MODEL

The aim of the Hub & Spoke Model workstream was to tackle the necessity of reconsidering traditional approaches to bleeding disorders care, focusing on adapting to new therapeutic options and advancing technologies. Workstream members identified two interconnected issues, which are still being dealt with both within and outside the EHC Think Tank.

The first issue, addressing immediate concerns about the delivery of gene therapy, will now entail collaborations between European medical and patient organisations to support national implementation and cross-border treatment. The second issue, concerning long-term considerations for future care, was transferred to the Future Care Pathways workstream. As a result, the Hub & Spoke Model Workstream was discontinued in February 2023.

ehcthinktank.eu/workstream/workstream-two

ADVOCACY AND AWARENESS

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

Partnerships

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

ERN-EuroBloodNet

eurobloodnet.eu

European Association for Haemophilia and Allied Disorders (EAHAD)

www.eahad.org

European Association for the Study of the Liver (EASL)

easl.eu/patient-synergies

European Hematology Association (EHA)

ehaweb.org

European Patients' Forum (EPF)

www.eu-patient.eu

EURORDIS-Rare Diseases Europe

www.eurordis.org

Platform of Plasma Protein Users (PLUS)

plasmausers.org

Transform Alliance

transformalliance.eu

World Federation of Hemophilia (WFH)

www.wfh.org

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.

New Technologies Workshop

3-5 NOVEMBER 2023

In 2023, the EHC Workshop on New Technologies in Haemophilia and Other Rare Bleeding Disorders gathered 70 participants in Cascais, Portugal, from 3 to 5 November.

This event is key to providing EHC members with education and information on the latest developments in novel therapies through thought-provoking presentations and patient/clinician panel discussions.

In addition to the sessions on replacement and non-replacement therapies, as well as gene therapy, the attendees discussed updates on novel therapies with the EHC Novel Treatment Review (NTR) Committee. Also, a session on considerations of treatment options was held.

The treatment and care of people living with extremely rare bleeding disorders was discussed. In terms of the organisation of care, sessions on shared decision-making and financial considerations regarding different treatment options were held.

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

NEW TECHNOLOGIES
IN RARE BLEEDING DISORDERS
WORKSHOP

3 - 5 NOVEMBER 2023
PESTANA CASCAIS HOTEL, PORTUGAL
www.ehc.eu

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 Health Economics Course

4-14 DECEMBER 2023

Even more accessible, even more inclusive!

For over a decade, the EHC has been providing its NMOs and a wider community with education on the evolution of health economics, tender process, and procurement in bleeding disorders.

In 2023, the EHC decided to change the format and switch from a face-to-face workshop to an online course on health economics in Europe to ensure access to education for patients and patient organisations on-demand for engaging in Health Technology Assessments (HTA).

The course provided online educational modules, homework and two live Q&A sessions with prominent experts to give guidance on being involved in HTA at any level. It covered HTA, cost-effectiveness and budget impact models in bleeding disorders, other payment models, and treatment updates in bleeding disorders.

Thus, 2023 was marked by the first edition of this online course – which was a success!

The course was positively assessed by 100% of the respondents! excellent (43,8%), very good (37,5%), and good (18,7%)!

The content of the course 2023 was evaluated positively by 93,8% of the respondents!

The EHC wishes to thank the Prime Global (HCD Economics) team, Mike Makris, 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, Cheryl Jones, and Claudia Mighiu.

93.8%

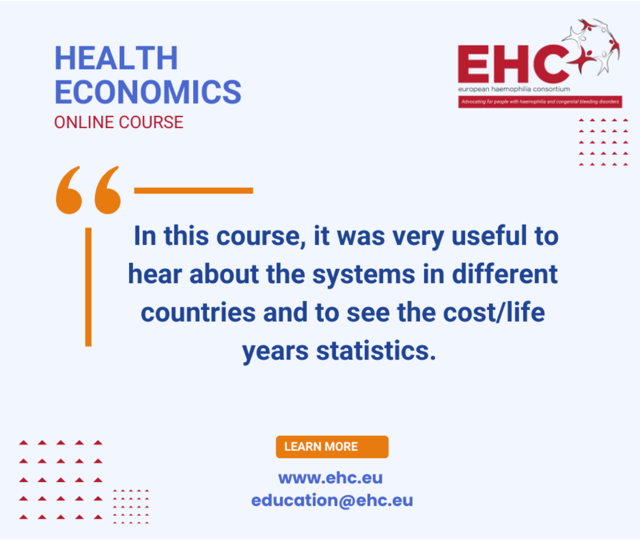
of respondents evaluated the course content positively

43.8%	**37.5%**	**18.7%**
Excellent	Very Good	Good

HEALTH ECONOMICS
ONLINE COURSE

In the EHC Health Economics Course 2023, I liked the breadth and depth of the concepts with practical relevant examples.

LEARN MORE
www.ehc.eu
education@ehc.eu



HEALTH ECONOMICS
ONLINE COURSE

In this course, it was very useful to hear about the systems in different countries and to see the cost/life years statistics.

LEARN MORE
www.ehc.eu
education@ehc.eu

HEALTH ECONOMICS
ONLINE COURSE

EHC Health Economics Course is when all concepts are in one place. Very well-explained definitions and their applications in our [bleeding disorders] field.

LEARN MORE
www.ehc.eu
education@ehc.eu

EHC Educational App EHCucate

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

In 2023, the EHC Educational App EHCucate continued to deliver trustworthy, impartial, and tailored to the needs of the bleeding disorders community education. While the treatment landscape of inherited bleeding disorders is evolving rapidly with complex therapeutic options, the EHC therapies educational app offers people with inherited bleeding disorders 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, soccer game and others) 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.

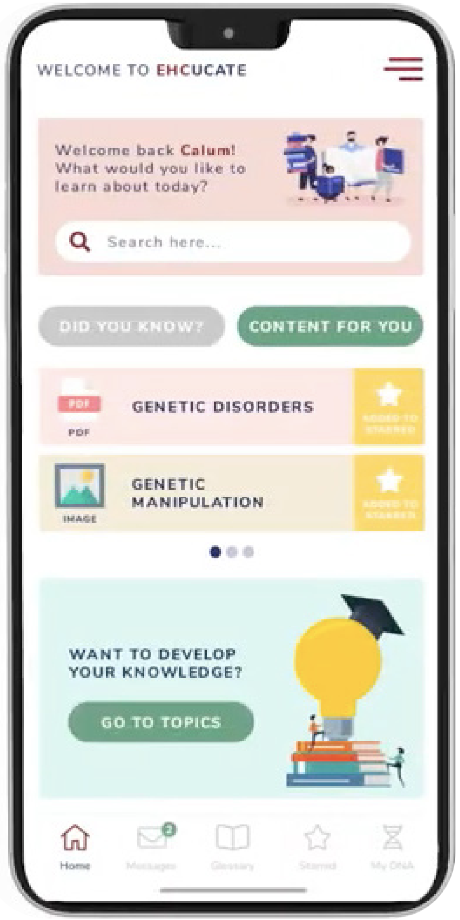
Explore Foundation, Non-Factor Replacement Therapies, Gene Therapy, and Replacement Factor Therapies topics in the app to learn about how blood clots and what the clinical trials are to the safety of the therapies, principles of factor replacement, and many more – all are customised and produced in-house by a team of dedicated medical writers, educational specialists and illustrators!

EHCucate is available for all and for free, both on Google Play (Android) and App Store (iOS). 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 back Calum! What would you like to learn about today?

WANT TO DEVELOP YOUR KNOWLEDGE?

Novel Treatment Review

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 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 EHC encourages its NMOs to use and adapt this document to their national needs but takes no responsibility for any changes.

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 periodically.

This document is overseen by the NTR Committee. In 2023, the EHC was not able to publish any issue and publication resumed in January 2024.

Novel Treatment Review Committee

The EHC is grateful for the work and support of the NTR Committee, which oversees the production of the NTR by supporting the EHC Staff in developing its content.

In 2023, the NTR Committee was composed of:

- **Paul Batty** | University College London, UK
- **Ana Boban** | EHC MAG, Croatia (Since October 2023)
- **Miguel Crato** | EHC President, Portuguese NMO (Since October 2023)
- **Mariëtte Driessens** | Dutch NMO
- **Radoslaw Kaczmarek** | Polish NMO
- **Ilmar Kruis** | Dutch NMO
- **Michael Makris** | Chair, EHC MAG, United Kingdom (Until October 2023)
- **Maria Elisa Mancuso** | EHC MAG, Italy (Since October 2023)
- **Declan Noone** | EHC President, Irish NMO (Until July 2023)
- **Brian O'Mahony** | EHC MASAG member, Irish NMO
- **Johannes Oldenburg** | EHC MAG, Germany
- **David Page** | Canadian Haemophilia Society
- **Flora Peyvandi** | EHC MAG, Italy (Until October 2023)
- **Uwe Schlenkrich** | Germany
- **Suthesh Sivalaparatnam** | Queen Mary University of London, UK

In addition to these volunteers, the EHC NTR is also edited by some EHC MAG and MASAG members.

Public Policy Review

The EHC periodic review of the European public policy landscape is entirely developed by the EHC to inform its NMOs about the rapidly changing landscape of health policy and legislative developments which have a direct impact on the bleeding disorders community.

Initially sent to the EHC NMOs in the form of a quarterly policy review, this informational product has recently been transformed into a periodic report and is available to readers on the EHC Academy website.

Consult the latest issues of the EHC Public Policy Review: **academy.ehc.eu/public-policy-review**

#ThisWay Campaign

In 2023, the EHC #ThisWay campaign continued its efforts to promote physical and mental health and well-being through various initiatives.

Throughout the year, the EHC shared three patients' stories to enhance understanding of the experience of individuals living with a rare bleeding disorder, as well as to provide insights into the caregiver perspective on the impact of such disorders on quality of life. Many of these patient accounts serve to inspire fellow patients to overcome challenges related to both physical and mental well-being.

In October, over two exciting weeks leading up to the EHC Conference, the EHC organised a virtual physical activity challenge for members of the community. Participants went above and beyond, tracking their physical activity with incredible enthusiasm, going beyond structured training sessions such as running and gym workouts to embrace a wide range of activities including walking, yoga, hiking, and more.

The EHC's motto throughout the challenge was simple yet profound:

'Every step counts'.

William's story

www.ehc.eu/williams-story

Ross's story

www.ehc.eu/ross-story

Scott's story

www.ehc.eu/scotts-story

EHC Youth Leadership Workshop 2023

EHC Youth Fellowship Programme

Building on the success of the Youth Fellowship Programme, in 2023 the EHC continued to support and mentor on a small group and individual basis many of our national and international participants on local and European projects to ensure the spirit of the Youth Leadership Alumni remain engaged and active within the community.

Connections continue to be created and restored with youth alumni participants through the EHC activities and events, actively encouraging our youth to continue their great work empowering young people with bleeding disorders in Europe.

EHC Youth Leadership Workshop

21-23 APRIL 2023

The EHC Youth Leadership Workshop took place from 21 to 23 April in Brussels, Belgium. This ninth edition brought together 16 participants from 15 countries.

The workshop contained sessions based on three core modules:

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

Participants also gain a valuable insight into the situationF regarding haemophilia and rare bleeding disorders care faced by the EHC NMOs in different countries from the information gathered in advance of the workshop by the participants.

An interactive and participatory approach was employed during the workshop, exploring active learning through icebreakers, interactive exercises, case studies and role plays.

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 youth training programmes and supervises EHC Youth Fellowship Programmes.

In 2023, the Youth Committee was composed of:

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

The work of the EHC Youth Working Group was supported by Fiona Brennan, EHC Patient Engagement Lead.

EHC Leadership Conference 2023

15-18 JUNE 2023

The EHC Leadership Conference is an essential element in strengthening the bleeding disorders community. This event brings together key figures of the EHC 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 2023, the Leadership Conference was held as a face-to-face meeting from June 15th to 18th in Brussels, Belgium. The programme as always is an opportunity to maximise and empower our NMOs to share experiences and best practices while also offering time to exchange on their challenges.

In 2023, the programme was based on the Principles of Good Organisational Health, a document that was adopted by the EHC GA and is the basis for the EHC NMO Organisational Health Programme (OHP). This approach provided more context to the OHP and helped to stimulate discussion between the NMOs.

Thematic areas of democratic structure, financial transparency and accountability, along with sustainable and formal relationships were all addressed and tackled with a variety of approaches. There was also a medical stream to help provide education and discussion amongst participants on developments in treatment.

The programme of the EHC Leadership Conference was developed by the EHC SC with the support of Kristine Jansone, EHC Community Development Lead, based on the conversations with the NMOs on their objectives and needs.

Women and Bleeding Disorders Committee

The objective of the EHC WBD Committee is to improve the quality of life of women and girls impacted by bleeding disorders. The WBD Committee creates awareness, recognition, support, and education about, and for, women in the bleeding disorder community at the European level.

In 2023, key activities included: a retreat for WBD Committee members in May, monthly virtual meetings, ambassador meetings via Zoom, and sessions at the EHC Leadership and Annual Conferences, focusing on diagnosis, care pathways, engaging women, and the importance of mental health and self-care.

The Committee shared two podcast episodes on the Principles of Care and prepared a movie for International Women's Day to raise awareness and demand better access to treatment, care and research for women and girls.

Listen to the EHC podcasts on SoundCloud:
soundcloud.com/european-haemophilia-consortium

Read more on the EHC marking International Women's Day 2023 here:
www.ehc.eu/the-ehc-marks-2023-international-womens-day

In 2023, the WBD Committee was composed of:

- **Marion Bräuer** | Austrian NMO
- **Yannick Collé** | French NMO
- **Karola Diósi** | Hungarian NMO
- **Jo Eerens** | EHC Vice-President Finance, Belgian NMO
- **Tatjana Markovic** | Serbian NMO
- **Ana Cristina Monteiro Pastor** | Portuguese NMO
- **Anna Tollwé (Chair)** | Swedish NMO

The work of this committee was supported by Zita Gacser, EHC Behavioural Change Lead.

Von Willebrand Disease Committee

2023 was a truly reframing year for the EHC VWD Committee. Set up as a working group in 2020 to lead the EHC VWD Platform for Europe, it was transformed into a committee in 2023 to reflect its evolution and development over the previous three years.

Same year, the Committee also completed its first term. Hence, the call for new volunteers was open to continue the work of increasing and improving diagnosis and treatment options nationally, and enabling VWD advocates all over Europe to work at the European level with other European partners towards improving access to treatment and care for all people with VWD.

In 2023, the VWD Committee was composed of:

- **Manon Degenaar-Dujardin** | EHC SC, Dutch NMO
- **Sunny Maini** | UK NMO
- **Julia Rauscher (Co-chair)** | Austrian NMO
- **Joanne Traunter (Co-chair)** | UK NMO
- **Baiba Ziemele** | Latvian NMO

The work of the Committee was supported by Daria Camilli, EHC Communications and Education Lead.

VWD Community Building and Support

The evolution of the EHC VWD Committee work and its relationship with the community prepared the ground for the switch from the concept of the VWD Ambassadors (EHC NMOs VWD representatives) towards the inclusion of each member of the European VWD Community as a whole in the decision-making, working together, and sharing ideas.

In June 2023, the EHC VWD Committee met in person to discuss the progress and make plans for the future. The work of the Committee and its existing and future projects were structured in three workstreams: awareness, engagement, education and research.

The community is now informally called #VWDunited and has its private WhatsApp chat which only VWD patients are invited to join.

Let's Talk VWD Series

- **April 24, 2023** Community call. The event was formatted as an open forum to talk about the challenges one faces when dealing with advocacy for VWD and the potential solutions to these issues. The Community call revealed the need to run the next meetings in the presence of a clinician who would address the most topical questions of the community.
- **September 27, 2023** Call with Dr Steve Austin, Clinical Director of Haemostasis at the Centre for Haemostasis - Haemophilia Centre, St George's University Hospitals. It was the first call to build the foundation of future progress in the strengthening collaboration between the European VWD Community and the community of clinicians.
- **December 4, 2023** Call with Prof Dr Frank Leebeek, Head of the Haematology department at Erasmus MC, the Netherlands. The audience had the opportunity to address all the questions about the genetics of VWD to Dr Leebeek.

Both meetings with clinicians were closed-door events for the EHC VWD Community only. The success of those and the identification of the needs served as an inspiration for the future collaborative project between the EHC VWD Committee and the ERN-EuroBloodNet which was launched on February 1, 2024.

Reach out to the EHC VWD Committee at their dedicated email address **vwd@ehc.eu**.

#VWDunited #VWDtogethere #beVWDaware

> “
>
> **Together we are stronger, our voice is clearer, and the change we make is experienced by more people in more countries. Together we can make a difference for those living with VWD all over Europe.**
>
> Jo Traunter, Co-Chair of the EHC VWD Committee

First-ever European VWD Awareness Day

On February 1, 2023, on the birthday of Erik Adolf von Willebrand, the European VWD Community marked its first Awareness Day. For the campaign, the EHC VWD Committee partnered with Agate Lielpētere, a Latvian visual artist, who shone a light on one of the primary VWD symptoms – bruising – and the psycho-social impact on all people with VWD.

The photography project How Did That Happen was specifically created to raise awareness of VWD and all those impacted and affected by the condition.

To know more about the project:
www.ehc.eu/vwd-awareness-day-2023

To know more about the artist:
www.agatelielpetere.com

European Rare and Inhibitor Network Committee

At the end of 2022, the term of the first IWG ended, giving the space for the ERIN Committee to continue its work in 2023.

Since 2023, then, the ERIN Committee has been responsible for developing, approving and helping to implement the content and the programme of the ERIN. The members of the Committee met in July 2023 to develop a strategic work plan to promote the ERIN Programme and embed people with extremely rare bleeding disorders at the European level, and work to support EHC NMOs to represent the needs of patients with rare bleeding disorders. The ERIN Committee developed a survey and a needs assessment to ensure they represent the patients and NMOs effectively.

The committee consists of people affected by extremely rare bleeding disorders, people affected by haemophilia A and B with inhibitors, as well as multidisciplinary care experts. The committee is supported by the EHC SC and Staff.

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.

In 2023, the ERIN Committee was composed of:

- **Christina Burgess** | United Kingdom
- **Miguel Crato** | EHC SC, Portuguese NMO
- **Ildiko Kaslik** | Hungarian NMO
- **Maria Elisa Mancuso** | Italy
- **Nathan O'Hagan Doyle** | Irish NMO
- **Jim O'Leary** | Ireland
- **Amy Owen-Wyard** | UK NMO
- **Maja Søndergaard Knudsen** | Danish NMO

The work of the ERIN Committee was supported by Kristine Jansone, EHC Community Development Lead.

The EHC is immensely grateful to all the members of the IWG for planting the seeds of the success of the ERIN.

SUSTAINABILITY

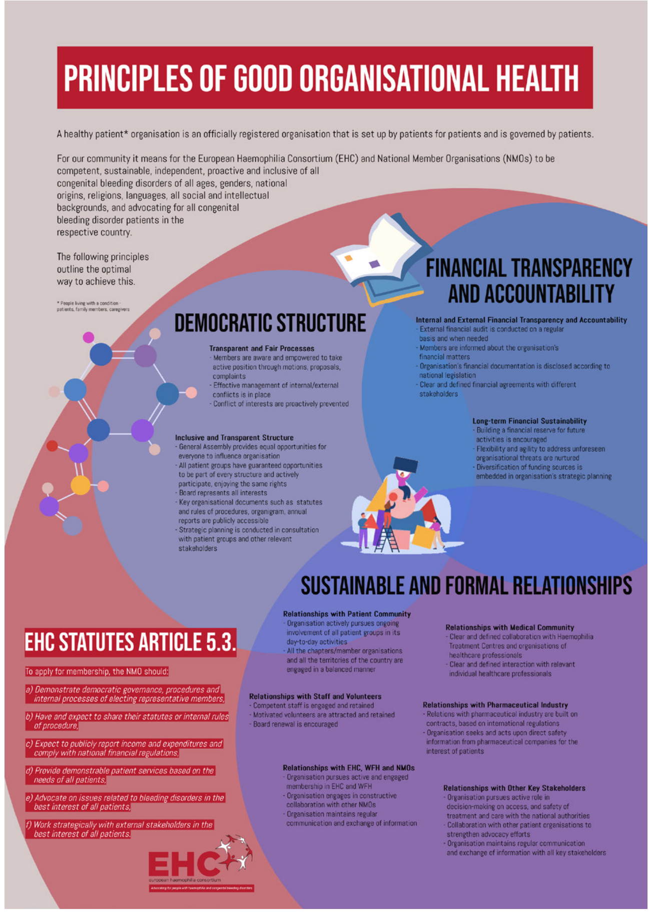
To ensure proof of EHC's organisational health andsecure its future development.

EHC Organisational Health Programme

The goal of the Organisational Health Programme (OHP) is to empower EHC 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 help them to remain healthy and well-functioning organisations. The plan to provide tangible, accessible and practical tools for our NMOs in areas of challenge remains a key objective of the EHC.

Building on the OHP which was approved by EHC members in 2022, the EHC 2023 OHP was designed to help EHC 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

In 2023, seven NMOs took part in an initial assessment offering a unique opportunity to reflect on their internal processes, their strategic vision, and areas of challenges they experience. The working group was supported by the Fiona Brennan, EHC Patient Engagement Lead, and Kristine Jansone, Community Development Lead.



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,

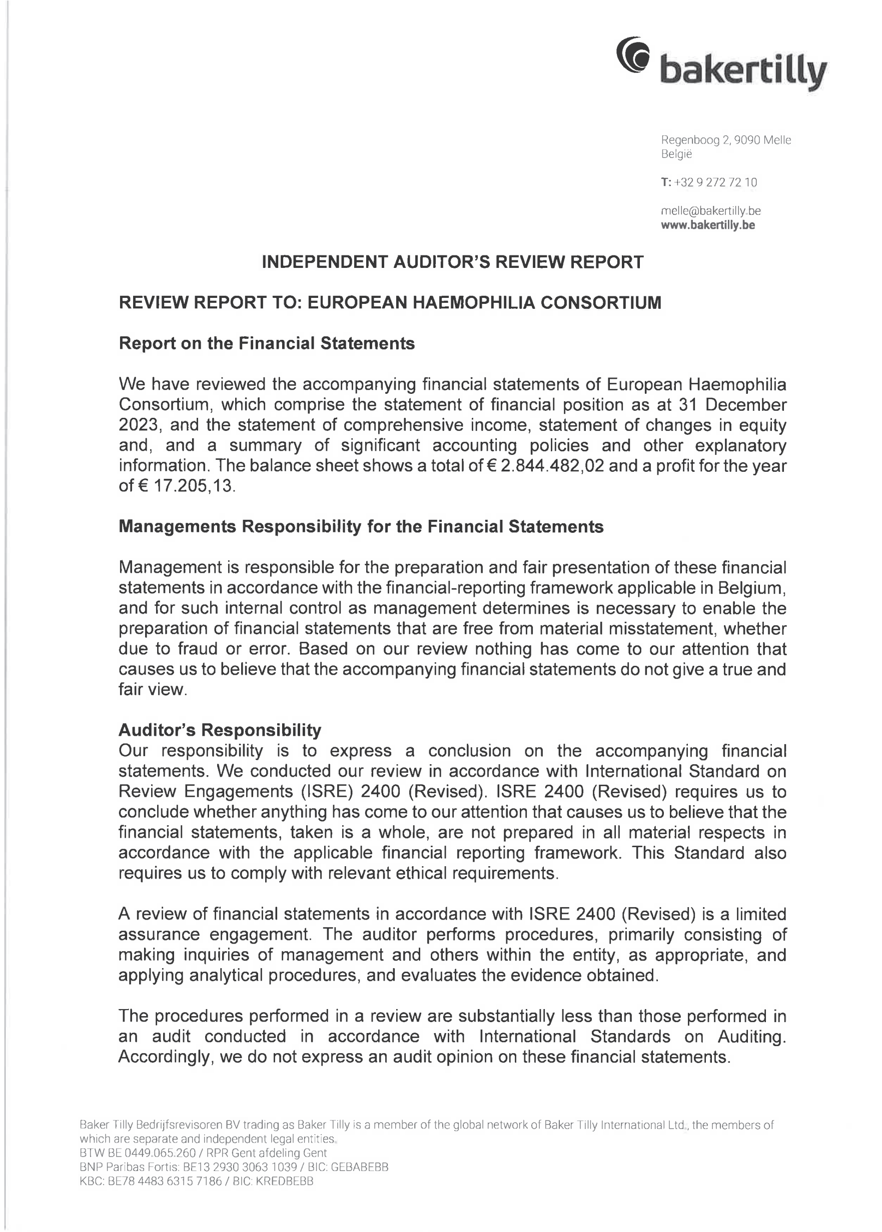
c) Expect to publicly report income and expenditures and comply with national financial regulations,

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.

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



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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 2023, 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 € 2.844.482,02 and a profit for the year of € 17.205,13.

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.

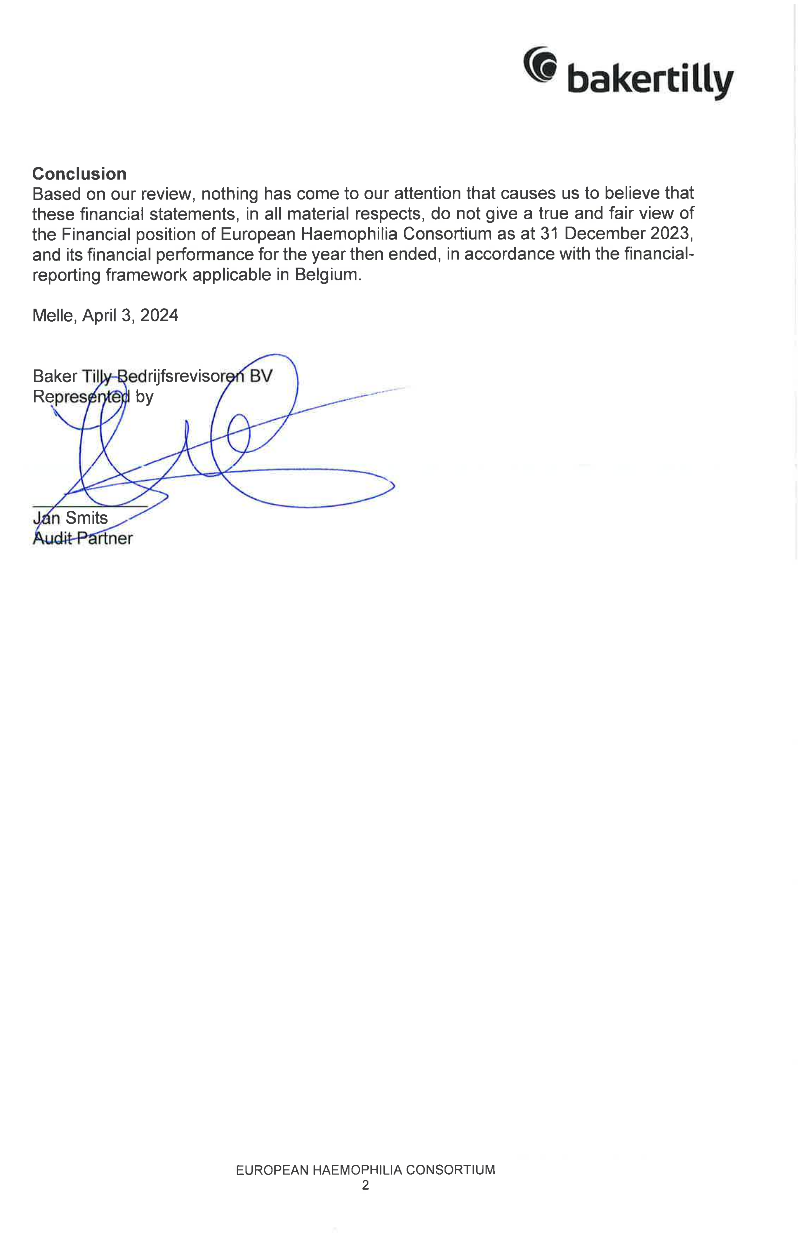
Auditor's Responsibility

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.
BTW BE 0449.065.260 / RPR Gent afdeling Gent
BNP Paribas Fortis: BE13 2930 3063 1039 / BIC: GEBABEBB
KBC: BE78 4483 6315 7186 / BIC: KREDBEBB



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 2023, and its financial performance for the year then ended, in accordance with the financial-reporting framework applicable in Belgium.

Melle, April 3, 2024

Baker Tilly Bedrijfsrevisoren BV
Represented by

Jan Smits
Audit Partner

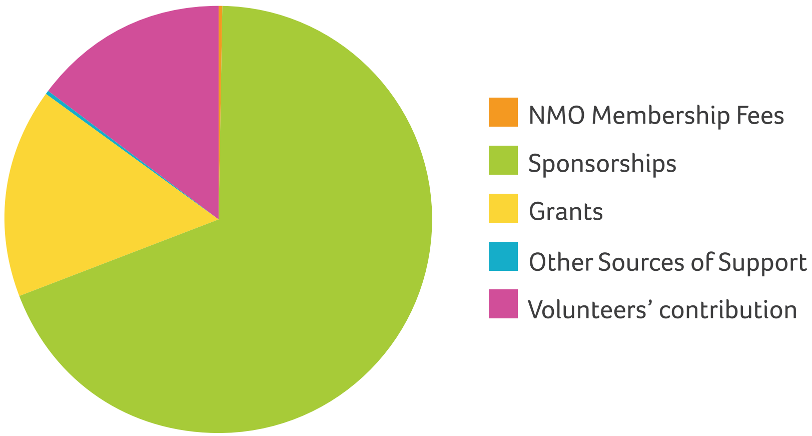
EUROPEAN HAEMOPHILIA CONSORTIUM
2

SUSTAINABILITY

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

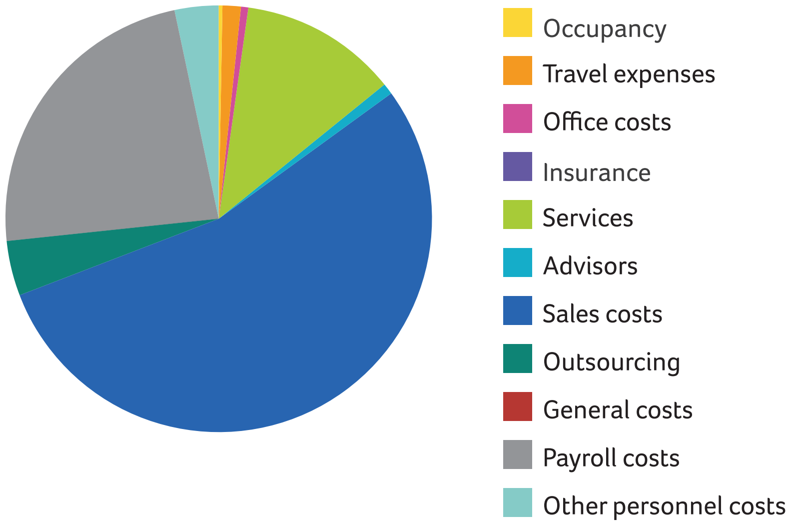
Income

INCOME	FINALS (€)
NMO Membership Fees	11,200
Sponsorships	1,876,857.69
Grants	430,000
Other Sources of Support	1900
TOTAL INCOME	**2,189,835**
Volunteers’ contribution	399,900



Operating Costs

EXPENDITURE	FINALS (€)
Occupancy	8,294
Travel expenses	29,804
Office costs	11,659
Insurance	2,947
Services	248,413
Advisors	20,183
Sales costs	1,144,203
Outsourcing	84,944
General costs	186
Payroll costs	493,804
Other personnel costs	68,477
TOTAL EXPENDITURE	**2,112,914**



BALANCE SHEET 2023
AS AT 31 DECEMBER 2023

ASSETS	Debit	%
Buildings and land	438,633	15.42%
Furniture, vehicles and equipment	7,828	0.27%
TOTAL FIXED ASSETS	**446,461**	**15.69%**
Amounts receivable within one year		
- Trade receivables	370,942	13.04%
- Other receivables	3,021	0.1%v
Current Investments		
- Other investments	189,319	6.65%
Cash and cash equivalents	1,799,141	63.25%
Transitory Assests	7,857	0.27%
TOTAL CURRENT ASSETS	**2,370,280**	**83.32%**
TOTAL ASSETS	**2,844,482**	**100.0%**

LIABILITIES	Credit	%
Equity	**2,224,168**	**78.19%**
Amounts payable within one year		
- Trade payables	267,238	9.39%
- Taxes, remuneration and social security	72,879	2.56%
TOTAL DEBTS	**340,117**	**11.9%**
Transitory Liabilities	**280,196.71**	9.8%
TOTAL LIABILITIES	**2,844,482**	**100.0%**

EHC SUPPORTERS
EHC 2023 Corporate Giving Partners

The EHC wishes to acknowledge and thank its 2023 corporate giving partners for making its work possible.

PLATINUM SPONSORS:

BioMarin | CSL Behring | Novo Nordisk | Roche | Sanofi | Sobi

GOLD SPONSORS:

Takeda | Pfizer | Regeneron | Spark | Hemab

SILVER SPONSORS:

Biotest | Centessa | LFB

The EHC also wishes to thank the following companies for supporting individual 2023 activities and projects:

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

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

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

The VWD Platform received support from Takeda.

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

Sobi and Pfizer kindly sponsored the EHC Health Economics Education and the Workshop on New Technologies in Haemophilia Care along with Sanofi.

The European Rare and Inhibitor Network couldn't have been carried out without the support of Roche, Pfizer, Centessa and Hemab.

The Think Tank activities were made possible by hands-off grants from BioMarin, CSL Behring, Novo Nordisk, Pfizer, Roche, Sanofi, Sobi, and Takeda. The 2023 Innovation Summit was kindly supported by BioMarin, Pfizer, and Sanofi.

Finally, the EHC 2023 Conference received support from Sobi, CSL Behring, Roche, Sanofi, BioMarin, and Pfizer as Platinum sponsors; Novo Nordisk as Gold Sponsor; and Takeda and Centessa as Bronze sponsors.

Volunteers

In 2023 the work of the EHC was supported by a total of 129 volunteers, who all together dedicated 5,665 hours of their time to the EHC.

Rezan Abdul-Kadir, Aizat Aidarbekova, Enea Atroce, Steven Austin, Marion Bräuer, Neil Bertelsen, Jan Blatný, Lara Bloom, Ana Boban, Simone Boselli, Steve Bourke, Christina Burgess, Raquel Castro, Dominik Cepic, Marie Louise Christiansen, Panagiotis Christoforou, Ellen Coeckelberghs, Donna Coffin, Yannick Collé, Massimo Colombo, Miguel Crato, Gregor Cuzak, Susan Daniels, Roseline D'Oiron, Piet de Kleijn, Manon Degenaar-Dujardin, Karola Diósi, Alison Dougall, Mariëtte Driessens, Marija Aloa Vera Drzmanoska, Jo Eerens, Carmen Escuriola-Ettingshausen, Jonathan Evans, Barbara Faganel Kotnik, Albert Farrugia, Enrico Ferri Grazzi, Karin Fijnvandraat, Giulia Gabrielli, Isabella Garagiola, Lorenzo Ghirardi, Paul Giangrande, Nicolas Giraud, Jenny Goudemand, Evelyn Grimberg, Richard Habis, Cedric Hermans, Anke-Peggy Holtorf, Dalma Hosszu, Claus Beck Jeppesen, Christian Jervelund, Matt Johnson, Mirko Jokic, Cheryl Jones, Gavin Jones, Sheba Joseph, Radek Kaczmarek, Ildiko Kaslik, Christine Keipert, Robert Klamroth, Konstantina Kostopoulou, Ilmar Kruis, Catherine Lambert, Vincenzo La Mura, Elise Lammertyn, Frank Leebeek, Diana Lighezan, Mark Lightowler, Sunny Maini, Mike Makris, Maria Elisa Mancuso, Charlotte Marchandise, Tatjana Markovic, Louise Mathieu, Evelien Mauser-Bunschoten, William McKeown, Nick Meade, Admir Mehić, Maia Meier, Claudia Mighiu, Flavio Minelli, Ana Cristina Monteiro Pastor, Lars Münter, Alexander Natz, Beatrice Nolan, Declan Noone, Cristina Novembrino, Nathan O'Hagan Doyle, Jamie O'Hara, Jim O'Leary, Brian O'Mahony, Johannes Oldenburg, Amy Owen-Wyard, David Page, Roberta Palla, Martine Pergent, Flora Peyvandi, Jim Phillips, Kelly Plueschke, Debra Pollard, Stefan Radovanovic, Priyanka Raheja, Julia Rauscher, Idaira Rodriguez Santana, Olivia Romero Lux, Frits Rosendaal, Uwe Schlenkrich, Roger Schutgens, Martin Sedmina, Suthesh Sivalaparatnam, Luigi Solimeno, Maja Søndergaard Knudsen, Sophie Susen, Marius Tanase, Renske ten Ham, Ana Toledo Chavarri, Anna Tollwé, Joanne Traunter, Nanda Uitslager, Sheela Upadhyaya, Tessa van Gastel, Thierry Vanden Driessche, Kristof Vanfraechem, Waander van Heerde, Mary Wang, Ingunn Westerheim, Laurence Woollard, 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
- Alexander Evtimov
- Art of e-Learning
- Brenda Cecilia Padilla Rodriguez
- CitrusSuite
- Conference Organisers
- David Mendes da Silva
- Elliott Laub
- Fiona Robinson
- Goran Kapetanovic
- Haemnet
- Han Han Li
- Jeff Stanford
- Julia Krivkina
- Lachezar Afrikanov
- Mark Brooker
- Matt Evans
- Netika
- Olga Miniuk
- Overlap
- School of System Change
- Svetoslava Stoyanova
- Weichie

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

Thank you for supporting the European bleeding disorders community!

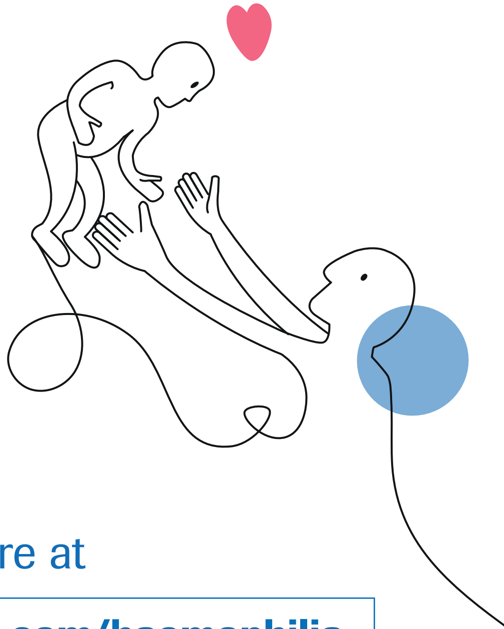
THANK YOU!

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

June 2024, Veeva document M-XX-00017597

LIBERATE
LIFE

Living with haemophilia may mean you experience symptoms such as reoccurring bleeds and joint pain, which can have an impact on everyday life.

But living with haemophilia doesn’t necessarily have to be that way.

Working with your care team to understand your level of protection against bleeds is a key step to improving your wellbeing.

Scan here to visit Liberate Life and find out how others have reimagined their story with haemophilia, or visit www.liberatelife.eu

sobi
rare **strength**

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Date of preparation: June 2024, NP-35513

We chase
the *miracles*
of science
to improve
people's lives

sanofi

www.sanofi.com

CSL Behring

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

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

EUR-OTH-0291 05/2024

Tradition and progress are in our DNA!

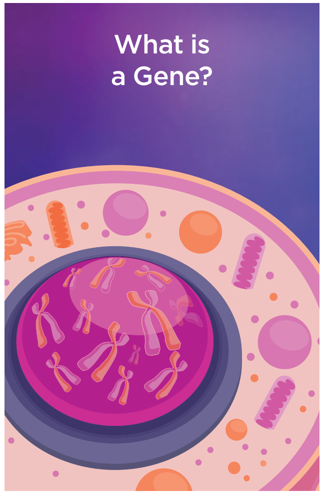
For over 50 years, we contributed significantly to major advancement in the treatment of bleeding disorders, such as haemophilia A / B and von Willebrand disease, with many of them being the first of their kind. Now and in the future we will continue to develop innovative and proven treatments that will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

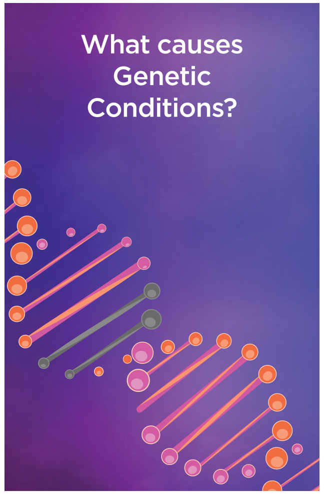
Learn differently.
Be involved differently.
HaemDifferently.

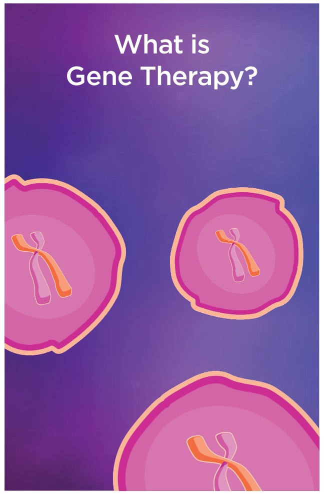
Haemdifferently
Presented by BIOMARIN

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:
www.haemdifferently.eu


What is a Gene?


What causes Genetic Conditions?


What is Gene Therapy?


How is Gene Therapy designed to work?


What are the goals of Gene Therapy Research?


What are the possible risks of Gene Therapy?

BIOMARIN

EUCAN-GTH-HEM-00234
Developed and funded by BioMarin | May 2024

We know that every haemophilia journey is different and requires solutions as unique as *you*

Isaac Miller
with his mother and sister
Lives in the UK and has haemophilia A

From addressing the key unmet needs to advocating for shared decision making when it comes to haemophilia care…

Together, we can work towards a ***better future*** in haemophilia.

Scan here to learn more and help raise awareness about the unmet needs that impact people with haemophilia

Date of preparation June 2024. HQ24CH00019

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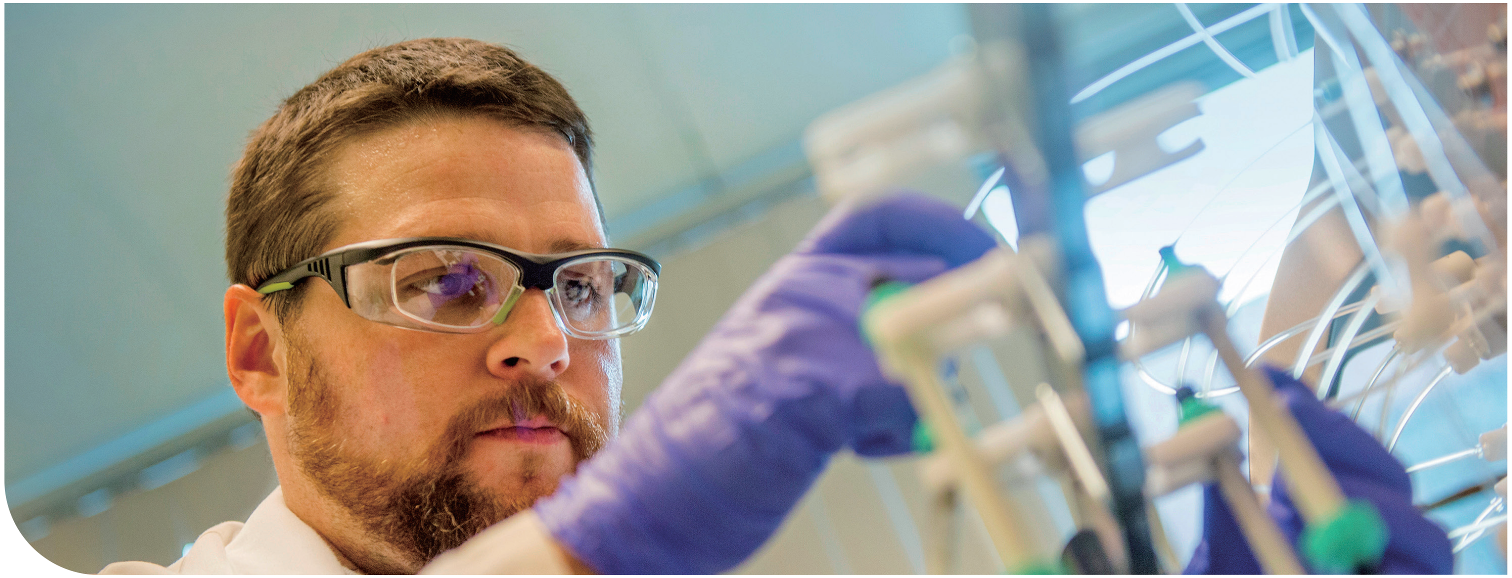

Better Health, Brighter Future

Takeda is a global, R&D-driven biopharmaceutical company committed to discovering and delivering life-transforming treatments and vaccines that have a lasting impact on society.

Since our founding in 1781 in a market stall in Osaka, Japan, our values endure by putting patient needs first, building trust with society, strengthening our reputation, and developing the business - in that order.

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Takeda

EHC

european haemophilia consortium

Advocating for people with haemophilia and congenital bleeding disorders

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𝕏 EHC_Haemophilia f European Haemophilia Consortium ◎ ehc_haemophilia

in European Haemophilia Consortium