EHC
european haemophilia consortium

ANNUAL REPORT
2020


Amanda Bok
EHC
Katalin Cseh
MEP
Declan Noone
EHC
Future outcomes
Simple answer:
Non-Replacement therapies
Bleeding is an adverse event and should be reported as one
Untreated bleeds
D-Dimers
Gene therapy
Factor level (One-stage vs chromogenic)
Liver function tests
Liver stiffness
Inhibitor testing
Steroid use (dose, duration,

ACRONYMS

CEO	Chief Executive Officer
EAHAD	European Association for Haemophilia and Allied Disorders
EASL	European Association for the Study of the Liver
EMA	European Medicines Agency
EPAG	European Patient Advocacy Group
EIN	European Inhibitor Network
EPF	European Patients' Forum
ERN	European Reference Network
HCV	hepatitis C virus
HTA	Health Technology Assessment
ISTH	International Society for Thrombosis and Haemostasis
IWG	Inhibitor Working Group
MEP	Member of the European Parliament
NMO	National Member Organisation
PARTNERS	Procurement of Affordable Replacement Therapies/ Network of European Stakeholders
PLUS	Platform of Plasma Users
UKHCDO	United Kingdom Doctors' Haemophilia Organisation
VP	Vice-President
VWD	von Willebrand Disease
WBD	Women and Bleeding Disorders


EHC
european haemophilia consortium
PLEASE CONNECT YOUR LAPTOP HERE
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Haemophilia and National registries
Haemophilia and National registries
Cedric Hermans
Cliniques universitaires

Caption?

INTRODUCTION

Needless to say that 2020 was like no other year: the COVID-19 pandemic affected our lives and habits, altered the well-established EHC activities, programmes and plans. However, the EHC quickly found new ways of working, to carry on community building, advocating, educating and empowering our members.

We rapidly adapted to the 100% virtual space and added a set of online resources and tools to our working environment to pursue our activities and bring our community members together online. It was also the first time that our annual Conference was organised virtually – while missing the energy and live interactions with our members, we were delighted to see a record high number of attendees. The virtual space brought unexpected advantages and enabled a larger outreach to the community. People can easily access our events and activities from the comfort of their homes and avoid the burden of long travel and tiredness.

2020 started with a major change for the EHC and the acquisition of an office in Brussels. This important step in the life of the EHC is not only the proof of EHC's organisation health and security for future development, but the acquisition of the new office also marks the settlement of the EHC into a more spacious and comfortable environment and will allow us to host events on the premises of the EHC.

ABOUT

EHC
30th anniversary
european haemophilia consortium

The European Haemophilia Consortium (EHC) is a non-profit, non-government organisation that works to improve the quality of life of people with haemophilia, von Willebrand Disease and other rare bleeding disorders in Europe.

The EHC was established in 1989 with the mission to:

- Ensure adequate supply – and access to – safe factor concentrate treatments,
- Improve diagnostic and treatment facilities,
- Promote patients' rights and raise ethical issues,
- Follow and influence developments in European health policy,
- Monitor the status of haemophilia care in member countries, and
- Stimulate research in all fields related to haemophilia and other 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.

"Just as we 'settled' into our permanent office space, we took the team and our work virtual. It wasn't always easy, but kudos to the stellar EHC team who not only never dropped a ball, but rolled with the changing times and re-invented how we serve our members; this beautiful annual report is a testament to our community of staff and volunteers!"

Amanda Bok, EHC CEO

"As our Annual Report shows, 2020 meant resilience and adapation, something the EHC and our patient community are resoundingly good at. It may not have been an easy year - for us and globally - but it fueled meaningful innovation that will help us come out even stronger."

Declan Noone
EHC President (2019-)

STRATEGIC OBJECTIVES

The EHC key role consists in shaping the quality of life of all people with congenital bleeding disorders in Europe, through partnership and advocacy: in substance, in form and into the future.

Our Strategic Objectives 2020 were:

1

ADVOCACY AND AWARENESS

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

- **World Haemophilia Week 2020**
- **EHC Conference**
- **Round Tables of Stakeholders**
- **New Technologies Workshop**

2

EDUCATION, ENGAGEMENT AND DEVELOPMENT

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

- Virtual Youth Leadership Workshop
- Youth Debate
- WBD webinars
- Economics Workshop

3

COMMUNITY EMPOWERMENT AND SUPPORT

To empower National Member Organisations and support their future development.

- EHC Leadership Conference
- Leadership Academy
- EURO 2020 Adapted Football & Physical Activity Camp
- Support to our NMOs

4

SUSTAINABILITY

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

- EHC Income And Expenditure 2020
- Independent Auditor's Report
- Balance Sheet 2020


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VIRTUAL YOUTH LEADERSHIP WORKSHOP

28–29 MAY | FIRST VIRTUAL EDITION

Notwithstanding the virtual setting, the philosophy of The Youth Leadership Workshop was not altered.

As in the face-to-face event, the aim of the virtual Youth Leadership Workshop 2020 was to bring together 16 youth participants from across our European NMOs, to exchange, learn and engage with each other for the first time at a European level.

The two-day online workshop of 5 hours focused on the three core modules of the Youth Leadership Programme:

1. **Fundamentals of patient organisations:** My NMO, My EHC, My Community;
2. **Medicines:** From discovery to market authorisation 10 step process & Industry-patient engagement;
3. **Volunteering:** Volunteering & Me

Throughout the workshop we employed a spectrum of interactive methodologies adapted to an online setting – these included lectures, panel discussions, group work and informal activities such as icebreakers.

As a constant transformation

- Break things
- Learning involves changing the brain
- Learning happens outside the classroom

As a Lifestyle

- Craft yourself consciously
- Learning is meaningful
- Learning is connected to the environment

As a Mindset

- I can learn anything, anytime
- Take responsibility of your own learning

YOUTH DEBATE

The Youth Leadership Workshop is also the moment to select four workshop candidates to take part in our much-loved EHC Youth Debate at the EHC Conference. The youth participants who were identified as part of the Youth Leadership Workshop 2020 to take part in the EHC Conference Youth Debate had the same opportunity as with previous years to avail of training and support prior to their debate. The debate was recorded and embedded as part of the EHC Conference platform and also included a networking evening on Tuesday 6th October where our EHC Conference delegates had the opportunity to speak with and ask questions to our Youth Debaters.

Listen to EHC 2020 Youth Debate Podcast
www.soundcloud.com/european-haemophilia-consortium/ehc-2020-podcast-youth-debate

EHC LEADERSHIP CONFERENCE

25-27 JUNE

The Leadership Conference is designed to bring together different national patient leaders and staff from the EHC's NMOs to exchange and engage on shared issues and questions.

Organising this important event for our community was a challenge given the virtual setting but in the end the online environment had the silver lining of offering participation to a more numerous and diverse group than the classical face-to-face version.

Taking place over three days with a daily programme of 2,5 hours the Leadership Conference 2020 focused on a variety of themes, employing a spectrum of methodologies adapted to an online setting.

The first day was dedicated to new developments in bleeding disorder treatment, both from a scientific point of view, as well as different NMOs' perspectives. The second day was dedicated to exchange of experiences and know-hows between NMOs, specifically addressing themes of regional cooperation, working with regulatory bodies and how to successfully coordinate work between different national patient groups. Finally, the last day was all about gene therapy, both from clinician and patient perspectives, and explored choice of treatment.

In addition to the core programme, each day featured an interactive session where participants could spend time together and engage in informal discussions. Icebreakers were part of each day's programme to increase energy, build a virtual atmosphere, and also help participants familiarize themselves with relevant online meeting features (e.g. polling). Finally, participants could also stay connected and engaged throughout the three days via a separate online social space (on padlet.com) where participants could continuously interact and share. With 80 registered delegates from more than 25 NMOs we were pleased to witness a significantly higher participation from youth in the online format; and a more regional participation from within an NMO's geographic membership than is typically possible with a three-delegate, in-person participation limit.

LEADERSHIP ACADEMY

13 AUGUST & 26 NOVEMBER

Given the virtualisation of our initiatives imposed by COVID-19, we felt important to offer our NMOs sufficient time and space to connect and discuss additional specific organisational issues, as well as providing them an opportunity to exchange among themselves and learn from each other's best practices. Therefore, we introduced the Leadership Academy – a workshop in the vein of the Leadership Conference but organised at a smaller scale.

With this new event on the EHC programme, we want to bring our NMO's continued insights and opportunities for a peer-to-peer exchange through a combination of quarterly online meetings with expert inputs, sharing of best practices and online materials. The first session took place in August and was dedicated to working with volunteers, focusing on various aspects of this collaboration (recruitment, retention and management). This first Academy brought together more than 40 participants from 20 NMOs, and was very well-received.

The second session was organised late November. The theme of the second session was "Patient Education: An NMO experience in bringing understandable education to our patients". Both leaders and patients shared their experiences and best practices on how to provide the most up to date and appropriate educational material to patients in an understandable and relevant manner. Time was also allocated to discussion on how we can help to achieve this in the European context.

WORLD HAEMOPHILIA WEEK 2020

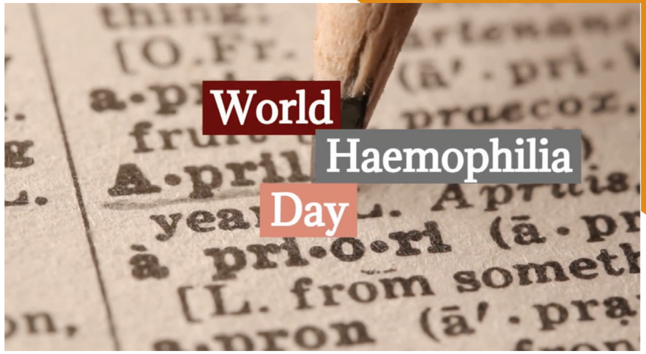
20 – 24 APRIL

In light of the ongoing COVID-19 pandemic, World Haemophilia Day 2020 was organised virtually and was spread out to a full week of online publications, advocacy and story-sharing.

The content of the EHC's virtual World Haemophilia Week (WHW) was varied in form and substance..

All content was published on a dedicated **EHC webpage** and shared on our social media channels. In addition to a video message shared by our President, Declan Noone, EHC World Haemophilia Week was marked by

- a presentation on The Importance of Patient Engagement and Community Involvement by Olivia Romero-Lux ;
- Brian O'Mahony, Prof. Flora Peyvandi and Declan Noone discussed Future Perspectives and Treatment Options in a video podcast ;
- a video-presentation of EHC Working Groups by Jim O'Leary; Naja Skouw-Rasmussen; Clive Smith; Manon Degenaar-Dujardin



MONDAY 20 APRIL

THE IMPORTANCE OF PATIENT ENGAGEMENT AND COMMUNITY INVOLVEMENT

Olivia Romero-Lux

TUESDAY 21 APRIL

FUTURE PERSPECTIVES AND TREATMENT OPTIONS

Brian O'Mahony, Prof. Flora Peyvandi and Declan Noone

TUESDAY 21 APRIL

EHC WORKING GROUPS

Jim O'Leary; Naja Skouw-Rasmussen; Clive Smith; Manon Degenaar-Dujardin

OPEN THE DOOR TO ANOTHER REALITY

EXHIBITION LAUNCH

World Haemophilia Week 2020 event culminated in the Introduction to EHC Exhibition “Open the Door to Another Reality” by Kristine Jansone and Amanda Bok.

The exhibition is marking the closing of the EHC’s 30th anniversary, rounding off the year of celebrations that started in April 2019 while also looking forward to our community’s future. As a result, the EHC created the exhibition “OPEN THE DOOR TO ANOTHER REALITY”, literally offering people the possibility to open a door and listen to the stories of people affected by bleeding disorders over different times and places across eight decades, and hear their hopes and visions for the future.

Originally foreseen to be launched on April 24th to coincide with the inauguration of the EHC’s new office, the exhibition is now awaiting our return to face-to-face mode, to be finally presented and attended by all! During World Haemophilia Week 2020, we shared a preview of what is to come: stories from the 1980s, a tumultuous decade for our community as well as the world. An excerpt from the virtual exhibition has also been made available, with the first 3 stories revealed.

OPEN THE DOOR TO ANOTHER REALITY

INTRODUCTION

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

WBD WEBINARS

28 & 29 MAY

In May 2020, the alternative to the face-to-face EHC Conference for Women with Bleeding Disorders was a pair of webinars organised online by the Women and Bleeding Disorders' Committee.

On May 28, the webinar focused on 'The management of heavy menstrual bleeding' and the following day was dedicated to 'The place of women in haemophilia comprehensive care centres.'

Both webinars received a positive feedback from the attendees, who rated then with an average of 4.5 out of 5 in terms of content.

28 MAY

Webinar on the management of heavy menstrual bleeding

126
participants

Management of heavy menstrual bleeding

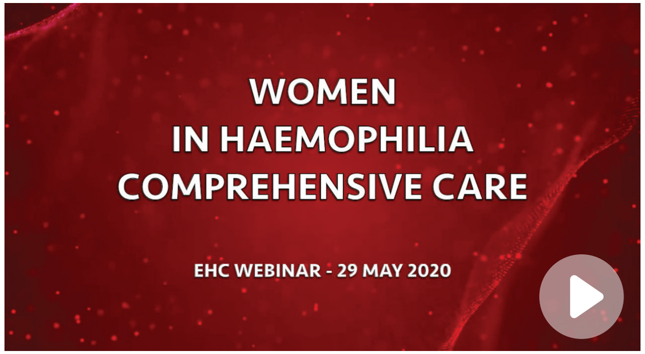
EHC WEBINAR - 28 May 2020

The first webinar on the management of heavy menstrual bleeding was attended by 126 participants, including EHC volunteers, patient representatives, healthcare professionals and industry. Participants came from 30 countries from Europe, North and South America, Asia and Africa. The event featured three presentations from healthcare professionals on explaining the physiological process of menses, managing heavy periods from a medical and psychological point of view and fertility and pregnancy when faced with heavy menstrual bleeding.

29 MAY

Webinar on the place of women in haemophilia comprehensive care centres

102
participants



WOMEN
IN HAEMOPHILIA
COMPREHENSIVE CARE

EHC WEBINAR - 29 MAY 2020

The second webinar was about the place of women in the haemophilia comprehensive care centre. Over 100 participants attended from around 30 countries and from various backgrounds. The event featured a presentation on the concept of comprehensive care, a discussion between a patient and healthcare professional on issues regarding women's inclusion in comprehensive care centres. The event concluded with case-studies on best practices to further include women in the comprehensive care centre.

ECONOMICS WORKSHOP

15, 17, 21, 24 SEPTEMBER

One of our strategic objectives is to ensure an adequate supply of - and access to - safe therapies. Therefore, for the past six years, we have been holding a workshop on health economics, which focuses on how medicines for haemophilia and other rare bleeding disorders are appraised and purchased.

We organise these 'Economics' Workshops in a three-year series to tailor the content to a specific region. In 2019 we launched a new cycle focusing on tender and procurement for haemophilia treatment in a landscape in which several therapeutic options ranging from traditional coagulation factor replacement therapy to gene therapy would co-exist. Therefore, in 2020, the focus was on the approach of the marketing authorisation of novel therapies such as gene therapy and the later clinical development of other non-replacement therapies. The 2020 workshop took place online in a series of webinars held during four two-hour sessions over two weeks and was attended by 50 participants from 18 countries in Western and Central Europe. The objective of the Economics workshop was to prepare participants to evaluate and compare different medical technologies that may arrive in their country. We want to train patients' representatives to fully engage in the tender and procurement process at the national level and give them the tools to advocate the best they can, whatever the national specificities of their healthcare system or procurement strategy.

From cesar garrido to Everyone
I can hear you

1: AN UPDATE ON NOVEL TREATMENTS

TUESDAY 15 SEPTEMBER

We dedicated the first session to inform participants on the latest developments in haemophilia A, B, inhibitor treatment and therapies for other rare bleeding disorders. This session also included tips on how to find information on medical and treatment developments.

2: SHARED DECISION-MAKING FOR OPTIMAL TREATMENT

THURSDAY 17 SEPTEMBER

The second session focused on treatment personalisation. We wanted participants to understand that different therapies may be suitable for different patients, depending on their medical history, fitness level, lifestyle, stage in their life, etc. A clinician and a patient shared their perspectives on treatment choice and shared decision-making. An economic model that could be personalised to each participant's country to perform an economic evaluation of treatment choices was also presented to the audience.

3: DEVELOP AN ECONOMIC MODEL FOR YOUR COUNTRY

MONDAY 21 SEPTEMBER

In the third session, the focus was on economics with a health economist's presentation on treatment choice and shared decision-making as well as group work on the model.

4: PAYMENT MODELS FOR NOVEL THERAPIES

THURSDAY 24 SEPTEMBER

In the fourth and final session, we featured a presentation on different pricing and procurement models, followed by a panel discussion on each model's pros and cons.

In conclusion, there were discussions on how to get involved in the national tender and procurement system. Participants were provided with a set of preparatory and explanatory material (video, audio, text) to get acquainted with the upcoming topics and notions and to use them after the workshop to spread the education among their community.

PRE-CONFERENCE WORKSHOPS

The EHC Conference gives EHC members a once-a-year opportunity to meet and learn about the latest scientific and medical information in the field of rare bleeding disorders. These meetings are also an excellent opportunity for EHC working groups and committees to meet and hold workshops on particular topics that they wish to work on with the broader EHC community. Despite its virtual format we still wished to continue this tradition and give our members the opportunity to further meet, exchange and work together on important topics.

NURSING

Nursing Care as part of a comprehensive care model in bleeding disorder treatment

SATURDAY 3 OCTOBER

This pre-conference event brought together nurses and selected patients from specific countries to share experiences and develop strategies to improve nurse-patient interaction in order to empower and facilitate learning for both the nursing community and patients. The workshop served as an excellent opportunity for regional nurses and patients from their centres to share experiences and develop strategies together to establish best practices.

YOUTH

EHC Youth Leadership Pre-Conference Day

SUNDAY 4 OCTOBER

Following the two-day Youth Leadership Workshop organized in May, we delivered a third Youth Leadership Workshop day as a pre-conference event. This session was dedicated to advocacy, youth engagement and EHC governance. This pre-conference workshop was attended by our Youth Leadership Workshop Class of 2020. The added benefit was undoubtedly to bring the group together for more team-building and also to enable us to fill in any gaps we felt had been missed during our initial meeting in May.

WOMEN & BLEEDING DISORDERS

Working together in the rare bleeding disorder community – The start of a conversation!

SUNDAY 4 OCTOBER

Although women are an integral part of the rare bleeding disorders community, they are not fully represented within the community and many still struggle to access diagnosis and care. With this workshop, the EHC Women and Bleeding Disorders Committee wanted to kickstart conversations on the role and place of women within this community.

INHIBITORS

Finding ways for better inclusion of the PWI in the life of the NMO

MONDAY 5 OCTOBER

People affected with inhibitor often struggle to feel included within the activities of their national member organisation, often due to the limited capacity of such patient associations. Through this pre-conference workshop, the EHC focused on providing specific knowledge and tools to its NMOs to better support people affected by inhibitors. In this year’s workshop, we looked more specifically at how to manage people with inhibitors during surgery and trauma, latest medical innovation and the state of inhibitor treatment and care in Europe.

VWD

Introducing the Von Willebrand Platform

WEDNESDAY 7 OCTOBER

In 2020, we started a working group for people affected by Von Willebrand Disease (VWD). During this workshop, EHC unveiled the results from its 2019 survey on VWD and looked at ways to better integrate this patient group within the work of national member organisations. The workshop also passed in review the recently issued VWD medical guidelines and how they can be better integrated within national care.

DENTAL

The life course approach to facilitate oral health for people with bleeding disorders

SATURDAY 10 OCTOBER

This workshop was organised for dentists with an interest or working with people affected by rare bleeding disorders. The event started with an introduction to rare bleeding disorders and how to treat people with these conditions, following with the multi-disciplinary risk assessment of dental procedures, prevention and management of periodontal disease, oral surgery for children and adults. Finally, several case studies were presented and discussed.

EUROPEAN HAEMOPHILIA CONSORTIUM
CONFERENCE 2020

Declan Noone
President, European Haemophilia Consortium

EHC CONFERENCE IN NUMBERS

1,085	chat messages sent
393	conversations
28	video calls
733	people logged in
93	countries attended
1,465	exhibition hall visits

EHC CONFERENCE

3-8 OCTOBER 2020

Just as all the other EHC events and workshops, the EHC 2020 Conference took place online from 3-8 October. The virtual setting was a success, both in terms of high attendance rates and in terms of positive feedback from the attendees. Registrations were free of charge for all the attendees.

The Conference was organized on a specifically dedicated online platform, where users could access the presentations, take part in the discussion on the events' chat or in a private chat with one another. A set of special EHC and Sponsors virtual rooms were also in place for participants to meet and discuss with the EHC President and CEO and reach out to sponsors. At the end of each day, participants could also join two networking spaces, either to meet the speakers of the day or to meet one of the EHC working groups representatives.

A series of podcasts complemented the varied and comprehensive Conference programme and all the presentations and documentation from the sessions were also made available on the platform. The Conference platform featured additional spaces for social interaction, such as the activity feed, where participants were invited to leave their posts and comment on posted material, all the EHC social media were also linked to the Conference Platform, this way amplifying the communication around the event. Finally, a special EHC 2020 musical playlist was shared on the Conference platform for all to enjoy the tunes selected by the EHC Community during the breaks.

This years' Conference programme was articulated around the following main themes:

- Pain
- Individualization in Bleeding disorders
- Mild-haemophilia and vWD
- Therapies on the horizon
- Education

FEEDBACK:

98%
of attendees rated the Virtual Conference as excellent

100%
of attendees said that the conference met or exceeded expectations

"An excellent conference!"

"Thanks for this week. It was amazing!"

"Thank you so much from the whole community, we needed a laugh!"

"Thanks to the organizers for a superb online experience."

"Thank you for the virtual conference. It was successful to maintain a link between us."

"The conference was great! I really enjoyed it. It is a great way to keep being updated and in touch with this amazing group of people. I really miss you all and hope to see you next time in real life. The chat was nice and well used. Great to also have some fun moments during it."

"It was amazing, inspirational, and also incredibly welcoming, at a time when being part of a community is so important for us all. I am so energised, motivated and inspired, and want to do even more, following your conference! This cannot be underestimated in terms of the ripple effect of your influence, guiding light and mentorship you provide to us through your important and invaluable work. The EHC is an incredible organisation and I am so proud to be a tiny part of it."

ROUND TABLES OF STAKEHOLDERS

The concept of EHC Round tables is really in the name : we bring together different stakeholders, people involved in haemophilia care like doctors, patients, industry, politicians and representatives of regulatory agencies around the table to have an open conversation on a particular issue. The event usually features some presentations or guided discussions with experts to inform the public about the subject. Then the floor is open for questions and answers, and conversations with all participants.

REGISTRIES AND CLINICAL OUTCOMES

18 FEBRUARY 2020

- Patient registries provide accurate data on patient numbers, prescribing patterns, geographical spread and adverse events, which is crucial for delivering proper treatment. However, many countries in Europe still do not maintain national registries on haemophilia while those who do are not utilising the same methodologies. This is why regulatory agencies and policymakers should support the establishment of national patient registries and decreasing discrepancies in data collection across countries.
- Data collection is at the core of establishing and maintaining efficient databases. Efforts should be made to ensure proper and high-quality data collection through the use of technologies, smart wearables and uniform data collection methodologies.
- The role of patients in maintaining effective patient registries should not be neglected. In Ireland, the so-called Patient Platform not only provides access to health data and interactive scheduling for patients, but it also ensures a way for them to include, edit and provide data to be included in the registry. Patient-physician collaboration is key for the future of clinical outcomes.
- Innovation is also a key aspect in this topic, especially in terms of novel treatments such gene and cell therapies where we still have only limited amount of available data. Precise data collection is fundamental to determine the efficacy and safety of treatments. This can be achieved through transferable databases across countries, the dynamic input of data from technologies such wearables, smart apps and smart scales, and through the use of predictive algorithms.

Marie-Laure Hecquet, EDQM

The event was kindly hosted by Member of the European Parliament Katalin Cseh.

ADVOCACY AND AWARENESS

On Tuesday 27th October, we organized two virtual Round Tables of Stakeholders on “Patient-Clinician Education Models” and “Gene therapy: where do we stand?”.

The online setting was perfectly fit for the event’s goal and format. Each Round Table gathered various profiles of stakeholders (patients, healthcare specialists, industry, academics) and allowed time for discussions and debates among the participants.

PATIENT-CLINICIAN EDUCATION MODELS

27 OCTOBER 2020

The objective of this roundtable was to outline the necessity of integrating a patient-clinician education approach into healthcare models, and give an overview of the benefits of a patient-as-partner engagement in disease and healthcare management. The event featured presentations and discussions. MEPs Stelios Kympouropoulos and Alex Agius Saliba took part in the event by expressing their support for the haemophilia community.

The roundtable sparked significant interest in further understanding the application of patient-clinician education models at grassroots level. Key concerns encircled around the lack of awareness observed amongst physicians in Europe on engaging with patients through this approach. Moreover, promoting patient engagement to the patients themselves was also foreseen as a hurdle, since they may not realise their full capacities and competencies within disease management and healthcare improvement. Considering these points, all participants agreed that a pragmatic and effective implementation of patient-partnership education models is needed to mobilise patients’ perspective into care. In summary, the following important points were highlighted during the event:

- The Montreal model provides a framework of co-accountability between patients and professionals with respect to disease management and improvement of healthcare services. Such an approach integrates the experiential knowledge obtained from patient-clinician partnership, and mobilises it for better treatment outcomes.
- Education models need to facilitate the transition of patients as passive care-recipients to care partners, which should be done through the proper dissemination of information to educate both physicians and patients alike
- Patient-doctor partnership in haemophilia care is a critical determinant of the quality of the disease management, therefore this collaboration should be addressed and included in the European principles of haemophilia care.
- It is essential for physicians to go beyond the patients’ medical diagnosis and treatment regime and perceive the patients’ lifestyles as a key components of disease management. Patients on the other hand, should realise the value of cooperating with the medical community in order to improve their own health. Efforts are needed on both sides.

GENE THERAPY: WHERE DO WE STAND?

27 OCTOBER 2020

This EHC Roundtable aimed to shed light on the benefits and pertinent challenges of gene therapy within the haemophilia community. In this regard, current and future implications were addressed, generating key considerations that would impact trajectory of this novel treatment. MEPs Manuel Pizarro and Katalin Cseh attended the roundtable, pledging their political support to the haemophilia community and advocating for more action at the European level.

Although a number of key considerations would need to be addressed, speakers estimated that potentially 10-20% of severe haemophilia A patients and 20-30% of severe haemophilia B patients may consider this treatment by 2030. In summary, the following important points were highlighted during the event:

- Although advances have been made in gene therapy, fundamental questions concerning reliability, variability, durability and safety need to be addressed. Nonetheless, these obstacles should not impede the licencing of gene therapies in the next years.
- More liver biopsies are essential for a better assessment of integration, host-response to the viral vector and processing of FVIII and FIX genes. Each company needs to take the responsibility in implementing and proceeding with regular and sufficient liver biopsies studies in each trial.
- Gene therapy licencing is taking on an evolving role within the EMA so as to guarantee patient access and protection whilst ensuring that innovation is also placed at the forefront.
- Systematic problems relating to affordability, notably budget impact and the cost vs value differential, can be potentially overcome with the creation of social contracts for patients.
- A two-way communication stream between medical professionals and patients is essential to ensure that any challenges are accounted for when considering or administering gene therapy.

NEW TECHNOLOGIES WORKSHOP

10, 12, 17, 19 NOVEMBER

over
80
participants

from
30
countries

With the rapidly evolving medical and scientific advances made in haemophilia, patient representatives must keep abreast with the latest developments.

One of the roles of the EHC is to help national patient organisations, and consequently patients themselves, make the most informed treatment choice. To this end, we have a series of tools to disseminate the latest scientific information, including the Workshop on Novel Technologies in Haemophilia and Other Rare Bleeding Disorders. This particular workshop is tailored for more experienced patient advocates with an advanced understanding of the novel medical technologies in development in this disease area. This is the only workshop in which industry representatives are not allowed to attend. This is to ensure that both participants and speakers can discuss freely about novel therapies without feeling scrutinised.

The virtual 2020 workshop was organised in four two-hour sessions held over two weeks, so that to cover all programme content and limiting screen-fatigue. Over 80 participants from 30 countries, primarily in Western and Central Europe, joined the event.

Our network of healthcare experts being extremely busy in their practice because of COVID-19 pandemic, we turned to pharmaceutical companies for updates on treatment options that we presented to our participants. Unfortunately, this was, unbeknown to the EHC, against local compliance rules, and therefore the EHC will not repeat this method. Each session featured data provided by companies (to the extent of the compliance rules allowing this) followed by a review from a healthcare professional who would complement data and answers questions from the public. Each session also featured a panel discussion on safety, efficacy, post-marketing surveillance, and the use of the technologies in different clinical scenarios (prophylaxis, surgery, breakthrough bleeds).

The workshop was dedicated on updates on extended half-life coagulation factor concentrates, non-replacement therapy and gene therapy and gene editing. The final session focused on the organisation of care, financial aspects and shared decision-making.

WORKSHOP
NEW TECHNOLOGIES IN HAEMOPHILIA
AND OTHER RBD

REPLACEMENT THERAPIES	**10 NOVEMBER**
12 NOVEMBER	**NON-REPLACEMENT THERAPIES**
GENE THERAPY	**17 NOVEMBER**
19 NOVEMBER	**ORGANISATION OF CARE**

- ALL SESSIONS WILL BE HELD IN ENGLISH,
- WORKSHOP OPEN NMOS ONLY,
- REGISTRATIONS DONE VIA YOUR NMO,
- REGISTRATIONS CLOSE ON 8 NOVEMBER.


BARRETSTOWN
LIVE
PRESS PLAY
RES 1080i
FPS 50
CODEC ProRes LT
00:00:00:00
TRIGGER REC Never
SOURCE SDI
CARD Ready

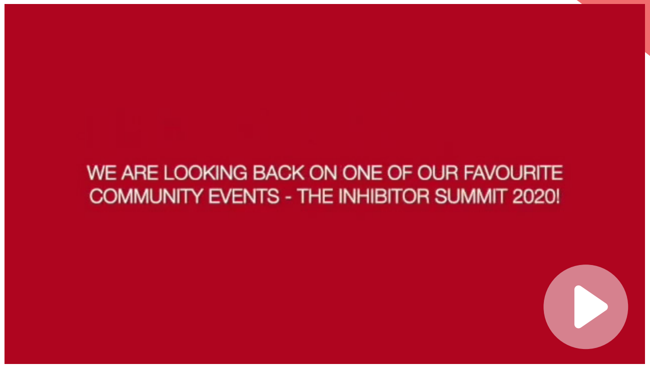
INHIBITOR SUMMIT 2020

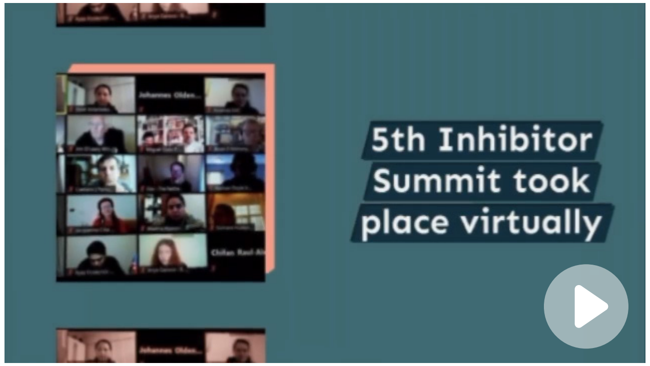
4-5-6 DECEMBER

Despite the online settings, the purpose of the Inhibitor Summit remained unchanged : we cared to offer a special time and space for people with haemophilia who have inhibitors, as well as their family members and caregivers, to build a community, to help to educate and empower themselves and to improve the quality of their lives.

Notwithstanding the limited possibilities of an online event in comparison to a real-life Barretstown experience, we have tried to accommodate as many elements of the programme as possible. The event included an update on the latest in inhibitor treatment, peer to peer sessions, discussions about quality of life and also family and kiddy time and challenges to have a wonderful time all together online.

The different activities were organized throughout the Summit days at specifically dedicated hours, this way allowing everyone to join the session of their choice. Every family received a parcel from Barretstown with all the necessary tools and ingredients to take part in the various group activities. Having the IS online enabled to extend the number of nominees, while in the face-to-face setting the number of attendees from each NMO was limited, due to logistical constraints.


WE ARE LOOKING BACK ON ONE OF OUR FAVOURITE COMMUNITY EVENTS - THE INHIBITOR SUMMIT 2020!



2020 MEANT

ADAPTATION & INNOVATION

With the challenging times imposed by the pandemic lockdowns and restrictions, we had to reinvent ourselves and the way we proceeded with our programmes and events.

EURO 2020 ADAPTED FOOTBALL & PHYSICAL ACTIVITY CAMP

One of the first activities that was adapted in the pandemic-driven context was the EURO 2020 Adapted Football & Physical Activity Camp, which was due to take place in July 2020. We did not want to lose all momentum and decided to bring physical activity, support and community building to our members virtually.

Over the course of four weeks from Tuesday 21st April and Thursday 23rd of April, we brought our EURO 2020 community together for a 30-minute physiotherapy, physical exercise or community building session online specifically for those members who requested to attend the EURO 2020: Adapted Football & Physical Activity Camp!

These virtual physiotherapy and physical exercise sessions were hosted by a physiotherapist with experience in working with people with bleeding disorders. In other sessions, the experts in our community engaged with our young members on relevant topics including providing facilitated peer support, providing tips and tools to stay well during the current restrictions.

#THISWAY

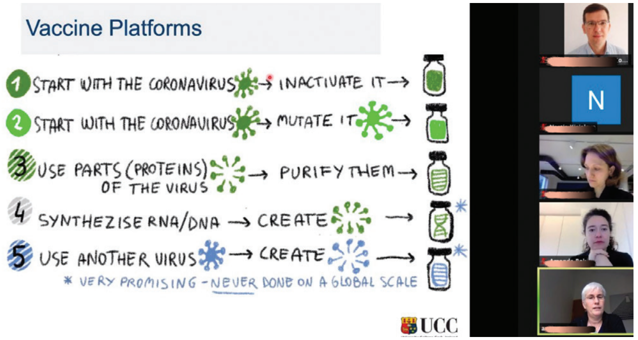
#THISWAY CAMPAIGN

In times of lockdown, physical activity and movement are more important than ever, not only for physical but also for mental wellbeing. This is why we decided to provide a space and guidance to our community to move and remain physically active from the comfort of their home. Therefore, the EHC took the #thisway campaign virtually. Every last week of the month up to the EHC 2020 Conference we organised virtual sessions of physiotherapy specially developed for people affected by rare bleeding disorders. These sessions were organized live on our Facebook channel and could be joined or re-watched by everyone at their convenience.

GUIDANCE ON PSYCHOLOGICAL WELL-BEING

During the difficult times of COVID-19 lockdown we tried to provide adequate guidance and support to our members on how to stay healthy, fit and keep their psychological balance intact. We published a special article with some useful advice and tips on how to stay mentally strong and confident despite the situation. We also followed on how our NMOs adapted to the constraints of the pandemic and came with innovative approaches (article).

Well-being in times of COVID-19
www.ehc.eu/ehc-now-discussions-on-psychological-wellbeing-in-times-of-covid-19


Vaccine Platforms

1. START WITH THE CORONAVIRUS → INACTIVATE IT →
2. START WITH THE CORONAVIRUS → MUTATE IT →
3. USE PARTS (PROTEINS) OF THE VIRUS → PURIFY THEM →
4. SYNTHEZISE RNA/DNA → CREATE → *
5. USE ANOTHER VIRUS → CREATE → *

* VERY PROMISING - NEVER DONE ON A GLOBAL SCALE

How we adapted to the COVID-19 crisis
www.ehc.eu/when-life-gives-you-lemons-or-how-we-adapted-to-the-new-reality-of-covid-19-crisis

COVID-19 COMMUNITY CALLS

Very soon after the onset of the COVID-19 pandemic we started organizing regular community calls for our NMOs with a specific focus on COVID-19 related questions and issues our members might have. During these calls we invited an expert to provide the latest update on the situation regarding COVID-19 and potential implications for people with rare bleeding disorders and also allocated specific time to questions and answers from the attendees. We also monitored the situation in all our NMOs to assess how the COVID-19 pandemic affected the activities of the national organisations and the treatment and care of the patients. The results of the survey were helpful in determining support actions and highlighting the needs for the community.

COVID-19 RESOURCES

We have created a dedicated space on our website with relevant and trustworthy resources and information on COVID-19, with a particular focus on rare bleeding disorders.

View COVID-19 resources
www.ehc.eu/coronavirus-updates/

NEW WORK TOOLS FOR EHC STAFF AND ADAPTATION TO A 100% VIRTUAL WORK SPACE

The EHC staff was forced to switch to a 100% virtual work space due to the COVID-19 situation. This also required finding some new ways of working and collaborating, as well for the operational team, as for the SC and WGs. Zoom became our meeting space and MURAL is our strong ally in the collaborative working space. The virtual setting didn't prevent us from continuing regular team-building activities and interacting with our colleagues and our members!

SUPPORT TO OUR NMOS

EHC Solidarity Fund
The EHC offered to cover the membership fees of NMOs in financial hardship.

EHC Activity grants and EHC physical activity grants
The EHC provides support options for NMOs who wish to implement certain activities but lack the financial resources.

2020 BROUGHT

NEW INITIATIVES

LAUNCH OF THE VON WILLEBRAND DISORDER EUROPEAN PLATFORM AND WORKING GROUP

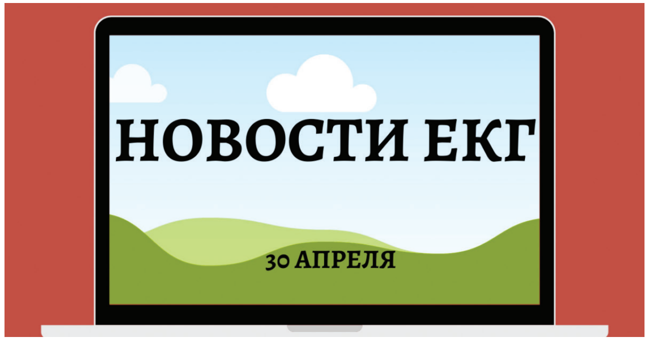
The EHC VWD working group was established in February 2020 to lead the work of the EHC VWD Platform for Europe. Since 2015, the EHC has made efforts throughout our various programmes and activities to address the gaps in the treatment and care of people with von Willebrand Disease. People with von Willebrand disease have often said that they feel isolated and that their disease, and its severity, can often times be overlooked or unrecognised. With this in mind, in order to take positive steps forward to improve the situation at a European level for patients with VWD, a more consolidated approach to the support, access to treatment and care was needed. For this reason, the EHC launched a VWD Platform for Europe, whose objectives are:

1. To promote the formation of a European community of people with von Willebrand Disease (PwVWD) under the auspices of the EHC enabling them to find support and 'identity';
2. To promote, build and maintain a network of VWD advocates in Europe;
3. To increase and improve diagnosis and treatment options nationally;
4. To enable those VWD advocates to work at European level together with other European partners (e.g. EAHAD, EMA, EDQM) towards improving access to treatment and care for all PwVWD.

"EHCUCATE", THE EHC EDUCATIONAL APP PROJECT ON NOVEL THERAPIES FOR RBD

In the past few years, the rare bleeding disorders community has been facing the emergence of novel and highly complex therapies. We believe that advocacy and education will be key elements for individual treatment journeys of our patients. This is why the creation of an educational and interactive app will be an essential tool towards a successful outreach to our community. To address the above, the EHC proposes the development of an educational app that would incorporate all levels of educational information about novel therapies, ranging from basic to advanced, and use a variety of in-app media to be engaging to a highly heterogenous audience. The project was launched in 2020 with a kick-off meeting and establishment of working groups in June.

The development of the educational app is still in progress. The launch of the beta version took place mid-May 2021 and the launch of the final version should follow by October 2021. EHCucate will be available for all and for free, both on Google Play (Android) and App Store (iOS).



EHC LAUNCHES RUSSIAN FRIDAYS – AN EHC-NEWS DIGEST

2020 started with a new communication project – the publication of EHC news and updates in Russian, to better serve the Russian-speaking EHC Community. Every Friday, we publish a compilation of our main relevant news in Russian, or share interesting articles and resources for our eastern-European members on our website and on Facebook.

EHC GENERAL ASSEMBLY APPROVES THE CREATION OF THE EHC THINK TANK AND COMMUNITY ADVISORY BOARDS (CABS)

In October, the EHC General Assembly approved the establishment of the EHC Think Tank - an initiative that thrives on reinventing how to do advocacy. The Think Tank will be a platform where a broad range of stakeholders will meet and collectively define the problems and the solutions with the ultimate goal to improve the quality of life for all with a bleeding disorder. This co-creation approach tackles long term change, and it has been used in other fields.

Faced with increased external demand for patient advisory boards, the purpose of the EHC-CABs is to provide an alternative to short-term, hand-picked industry advisory groups and to address questions and areas of common, community concern.

STEERING COMMITTEE (SC)

In January, the EHC staff and SC travelled to Copenhagen, the place where the EHC Conference was supposed to take place before COVID-19 altered our organizational plans, to meet for a strategic retreat. During this two-day retreat, the EHC held brain-storming sessions to define the values and assets of the organization, established and reinforced its strategic planning for the coming years, had team-building time and met in person with the organisers and service-providers of the planned EHC 2020 Conference.

The EHC 2020 SC members are:

Declan Noone
EHC President, Irish National Member organisation (NMO)

Minette van der Ven
EHC Vice-President Finance, Dutch NMO

Miguel Crato
EHC Steering Committee Member, Portuguese NMO

Naja Skouw-Rasmussen
EHC Steering Committee Member, Danish NMO

Thomas Sannié
EHC Steering Committee Member, French NMO

Amanda Bok
Ex-Officio Member, EHC CEO

Cesar Garrido
Ex-Officio Member, World Federation of Hemophilia

Following the GA of October 2020, the EHC SC was partially renewed.
We were saddened to part ways with Olivia Romero-Lux, Rodoslaw Kaczmarek and Michael Van der Linde.

Olivia Romero-Lux
EHC Steering Committee Member, French NMO

Rodoslaw Kaczmarek
EHC Steering Committee Member, Polish NMO

Michael Van der Linde
EHC Steering Committee Member, Dutch NMO

We are very delighted to welcome newly elected and co-opted members:
Stefan Radovanovic (Serbia) and Diana Lieghezan (Romania) as well as
Jo Eerens (Belgium), former EHC Community officer.

Stefan Radovanovic
(co-opted late 2020)
EHC Co-Opted Steering Committee Member, Serbian NMO

Jo Eerens (elected late 2020)
EHC Steering Committee Member, Belgian NMO

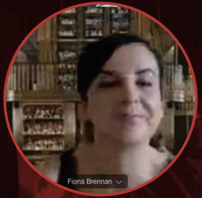
Diana Lieghezan
(co-opted late 2020)
EHC Co-Opted Steering Committee Member, Romania

STAFF

The daily work of the EHC is carried out by a Brussels-based team of dedicated staff members composed of Amanda Bok, Fiona Brennan, Thierry Hoppe, Kristine Jansone, Saskia Pfeyffer and Laura Savini.

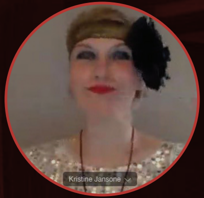
In January 2020 Nastassia Kisialeva joined the EHC team to work on Public Policy and Communications with Laura Savini. Nastassia is also leading the EHCucate project.

Amanda Bok



Fiona Brennan

Thierry Hoppe



Kristine Jansone

Saskia Pfeyffer

Laura Savini

Nastassia Kisialeva

MEDICAL ADVISORY GROUP (MAG)

The 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 Steering Committee and staff. The MAG is consulted on ad hoc matters and is a valued source of expertise and know-how.

In 2020 the MAG members were:

- **Prof Michael Makris** (Chairperson) | UK
- **Prof Flora Peyvandi** | Italy
- **Dr Jan Blatný** | Czechia
- **Prof Johannes Oldenburg** | Germany

MEDICAL AND SCIENTIFIC ADVISORY GROUP (MASAG)

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

Members of the MASAG are: Rezan Abdul-Kadir, Massimo Colombo, Piet De Klein, Roseline d'Oiron, Alison Dougall, Escuriola-Ettinghausen, Albert Farruggia, Dan Hart, Anneliese Hilger, Vince Jenkins, Radoslaw Kaczmarek, Evelien Mauser-Bunschoten, Giuseppe Mazza, Beatrice Nolan, Cristina Novembrino, Jamie O'Hara, Brian O'Mahony, Debra Pollard, Frits Rosendaal, Glenda Silvester, Luigi Solimeno, Thierry Vanden Driessche

NEW PRODUCTS COMMITTEE

The haemophilia treatment landscape is fast evolving with many novel and innovative therapies in the final stages of development or being marketed in Europe. To help our members navigate all of these changes, the EHC has developed a publication, the New Product Review, issued twice a year with the latest therapeutic developments. The members of the New Products Review Committee support EHC staff in developing the content of this newsletter.

The members of the New Products Committee are:

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

In addition to these volunteers, the New Product Review is also edited by some of the EHC MAG and MASAG members. The work of this committee is supported by Laura Savini, EHC Communications and Public Policy Officer.

INHIBITOR WORKING GROUP

The Inhibitor Working Group coordinates the activities of the European Inhibitor Network. This is a group of volunteers, consisting of patients, health care professionals and patient organisation representatives. The IWG is in charge of overseeing the EIN programme, and it collaborates closely with EHC NMOs. The Inhibitor Working Group met in February to define their strategic goals for the next three years and is composed of the following volunteers:

- **Enea Atroce** | Swiss Haemophilia Society
- **Christina Burgess** | UK
- **Miguel Crato** | EHC Steering Committee, Portugal
- **Mirko Jokic** | Serbian NMO
- **Ioana Malita** | Romania
- **Dr Maria Elisa Mancuso** | Italy
- **Jim O'Leary** | Irish NMO
- **Teresa Pereira** | Portuguese NMO

The work of the EHC IWG is supported by Kristine Jansone, EHC Community Programmes Officer.

YOUTH COMMITTEE

The EHC Youth Working group provides strategic guidance to the EHC on its Youth Programme. The Youth Working Group also develops and delivers the agendas and content of EHC's youth training programmes and supervises the EHC Youth Fellowship Programmes. In 2020 the EHC Youth Working Group is composed of:

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

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

THE VON WILLEBRAND DISEASE WORKING GROUP

The von Willebrand Disease (VWD) working group was set up in 2020 to lead the 'EHC VWD Platform for Europe.' This platform will promote the formation of a European community of people with Von Willebrand Disease (PwVWD) enabling them to find support and 'identity' while also promoting, building and maintaining a network of European VWD advocates. The Working Group will also aim to increase and improve diagnosis and treatment options nationally and enable those VWD advocates to work at the European level and other European partners towards improving access to treatment and care for all PwVWD.

The EHC VWD Working Group is composed of:

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

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

WOMEN WITH BLEEDING DISORDERS COMMITTEE

The aim of the Women and Bleeding Disorders (WBD) Committee is to gather people with a keen interest in promoting and raising awareness about issues related to women and bleeding disorders. Hence the role of the EHC WBD Committee is to create awareness, recognition, support and education about, and for, women in the bleeding disorder community at the European level. The Committee has been very active in sharing useful resources for the WBD community, some of which can be found on the EHC website dedicated page: **www.ehc.eu/bleeding-disorders/women-with-bleeding-disorders**

The EHC Women and Bleeding Disorders Committee met in March 2020 in Copenhagen for its strategic retreat to plan for the next three years of work. The committee for 2020-2022 is composed of 9 volunteers:

- **Yannick Colle** | French NMO
- **Evelyn Grimberg** | Dutch NMO
- **Diana Lieghezan** | Romania
- **Tatjana Markovic** | Serbian NMO
- **Ana Pastor** | Portuguese NMO
- **Katja Peltoniemi** | Finnish NMO
- **Naja Skouw-Rasmussen** | Chair of the Committee, EHC Steering Committee, Danish NMO
- **Anna Tollwé** | Swedish NMO
- **Sunni Maini** | UK NMO

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

OUTREACH

NOVEL TREATMENT PRODUCTS NEWSLETTER (NPR)

Our periodic review of novel treatments in haemophilia and other bleeding disorders is a newsletter entirely developed by the EHC to help educate EHC NMOs about the rapidly changing landscape of medicinal treatment in rare bleeding disorders. The newsletter 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 newsletter is compiled from multiple sources, including presentations at recent scientific meetings, websites, financial information and by writing directly to pharmaceutical companies. It is then redrafted and presented in easy-to-understand language. This newsletter is updated periodically. The EHC greatly welcomes all treatment developments that may benefit patients in the future. The EHC takes no position on any product type or class reported in this newsletter. In 2020, we issued two NPRs, including one issue in Russian.

Issue 1 (June 2020)

Issue 2 (November 2020)

HAPPY 1/2 HOUR WITH AN EXPERT IN HAEMOPHILIA CARE

In 2020 the EIN continued to organize monthly events with multidisciplinary experts in haemophilia care, with 8 virtual meetings held during the year. These events are a unique opportunity for the members of the inhibitor community to ask questions to experts in one of the areas of multidisciplinary care – haematology, physiotherapy, nursing care or psychosocial care – from the comfort of their home in a small group of peers.

MEMBERSHIP

In 2020, the organisations here below were part of the EHC membership:

Albania	Shoqatës Shqiptare të Hemofilikëve
Armenia	Armenian Association of Hemophiliacs
Austria	Österreichische Hämophilie Gesellschaft (ÖHG)
Azerbaijan	Hemofiliyalı Xəstələrin Respublika Assosiasiyası
Belarus	Белорусская Ассоциация Больных Гемофилией
Belgium	Association de l'Hémophilie - Hemofilievereniging (AHVH)
Bosnia and 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	Kyrgyz Haemophilia Society "Community of handicapped – haemophiliacs"
Latvia	Latvijas Hemofilijas Biedrība
Lithuania	Lietuvos Hemofiljos Associja
Luxembourg	Association Luxembourgeoise des Hémophiles
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	Republic of Tajikistan Public Organisation "Hemophiles"
Turkey	Türkiye Hemofili Derneği
Ukraine	Всеукраїнське товариство Гемофілії
United Kingdom	The Haemophilia Society
Uzbekistan	Узбекское общество гемофилии

PUBLIC POLICY CORNER

Once again, the public policy and advocacy stream of EHC was buoyant in 2020. We have been closely monitoring the COVID-19 situation and all the available medical and logistical information related to treatment for rare bleeding disorders and issued a number of important statements and publications for our community.

COVID-19

EHC-EAHAD JOINT STATEMENT ON HOME DELIVERY OF TREATMENT

Together with the European Association for Haemophilia and Allied Disorders (EAHAD) we released a joint statement in early April calling on the importance of home delivery of treatment during the COVID-19 pandemic so that patients avoid frequent hospital visits. The statement also highlights the importance of preparedness for any disruptions in the treatment supply chain.

View Full Statement here

TREATMENT OPTIONS

EHC RELEASES STATEMENTS ON DESMOPRESSIN NASAL SPRAYS AVAILABILITY

Following the international recall of all Ferring Pharmaceuticals Limited nasal sprays containing desmopressin in July 2020, we haven't stopped monitoring the situation to inform our community of the potential consequences and impacts on the users of these products, as shortages over the course of 2020 were expected.

View Full Statement here

WFH, EHC, NHF JOINT STATEMENT ON GLOBAL DOSING HOLD IN FITUSIRAN TRIALS INITIATED BY SANOFI GENZYME TO INVESTIGATE NEW ADVERSE EVENTS

In November, the WFH, EHC and NHF have learned of and subsequently confirmed a decision by Sanofi Genzyme to initiate a voluntary sponsor-led global dosing hold on its full clinical development program for fitusiran due to the identification of new adverse events.

View Full Statement here

GENE THERAPY

EHC-EAHAD JOINT STATEMENT ON HUB-AND-SPOKE MODEL FOR GENE THERAPY APPLICATION

In May, we addressed the preparedness for gene therapy in a joint statement with EAHAD- on promoting hub-and-spoke model for the treatment of haemophilia and rare bleeding disorders using gene therapies.

View Full Statement here

EHC-EAHAD RELEASE JOINT STATEMENT ON AAV8-BASED ASPIRO CLINICAL TRIAL

Late June, another important statement related to gene therapy trials was issued jointly by us and EAHAD, following two fatalities in a clinical trial using AAV8-based vectors in another rare disease area, XLMTM. It was paramount to provide further clarification on the situation, highlight key differences between the conditions studied as well as the gene therapies studied in the two disease areas.

View Full Statement here

STATEMENT ON UNIQURE'S GENE THERAPY CLINICAL PROGRAMME, HOPE-B, PUT ON HOLD FOLLOWING SUBMISSION OF A SAFETY REPORT

Late December, we informed our members of the hiatus in uniQure's gene therapy clinical trial for haemophilia B.

View full report here

EHC SIGNS MANIFESTO FOR A EUROPEAN HEALTH UNION

The EHC signed the Manifesto for a European Health Union. This initiative calls the political leaders of Europe to commit to creating a European Health Union to build a health policy that contributes to the long-term sustainable development of the European Union.

The Manifesto sets out a vision of a European Health Union (with goals, policies, measures and principles) developed by the signatories of the Manifesto to build a union where all people are as healthy as they can be throughout their lives.

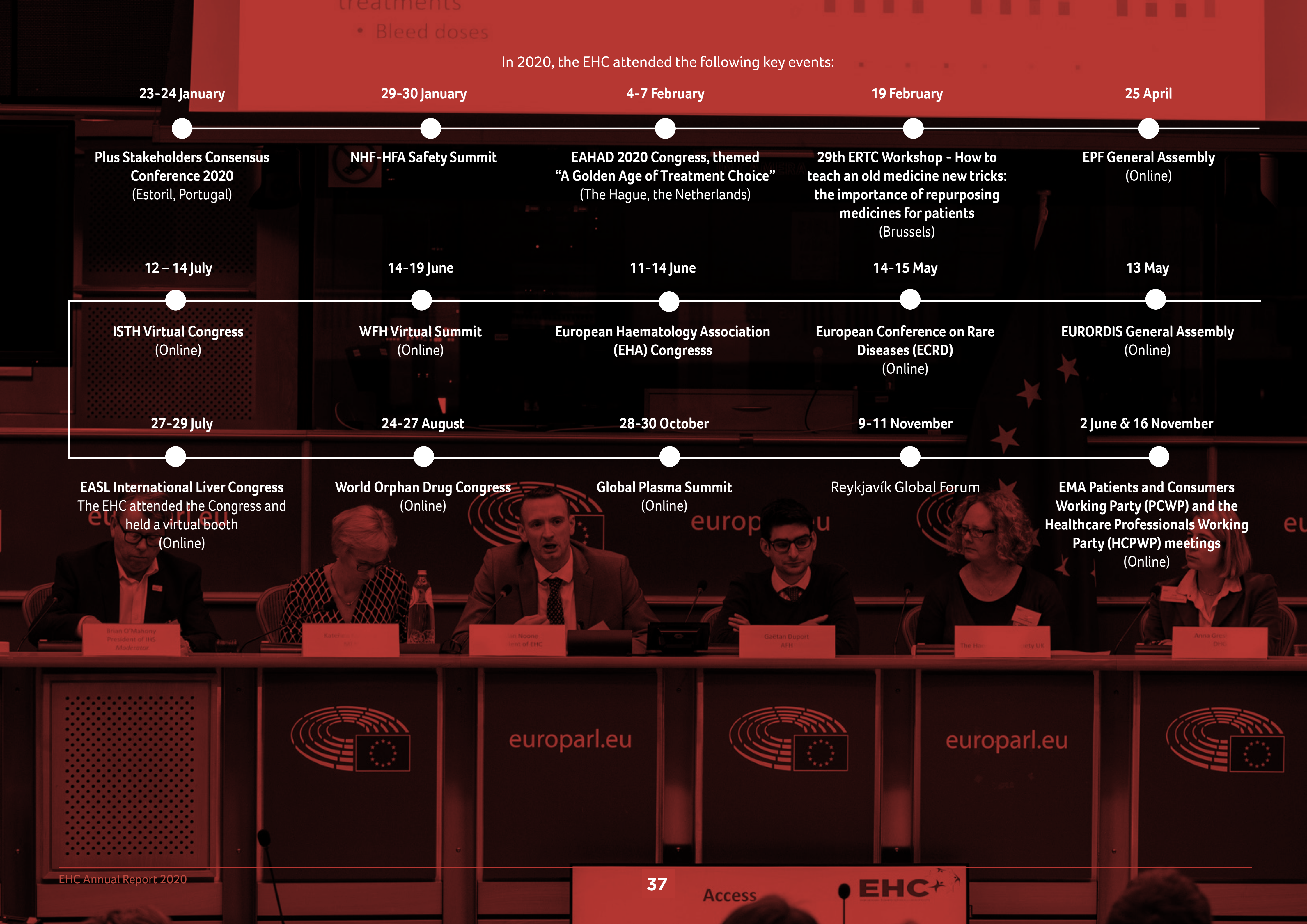
QUARTERLY REPORTS

One of the main EHC public policy publications is the quarterly report, which is a compilation with all the relevant events and developments on the political and advocacy fields with a special focus on rare bleeding disorders. This report is released quarterly and is shared with EHC NMOs and relevant stakeholders.

REPRESENTATION AND PARTNERSHIPS

The EHC works closely with key European and international partners, including other patients' groups and medical associations:

World Federation of Hemophilia (WFH)	European Association for Haemophilia and Allied Disorders (EAHAD)	European Organisation for Rare Diseases (EURORDIS)	Platform of Plasma Protein Users (PLUS)	European Patients Forum (EPF)	European Association for the Study of the Liver (EASL)
www.wfh.org/en	**www.eahad.org**	**www.eurordis.org**	**plasmausers.org**	**www.eu-patient.eu**	**easl.eu/patient-synergies**



In 2020, the EHC attended the following key events:

23-24 January
Plus Stakeholders Consensus Conference 2020
(Estoril, Portugal)

29-30 January
NHF-HFA Safety Summit

4-7 February
EAHAD 2020 Congress, themed “A Golden Age of Treatment Choice”
(The Hague, the Netherlands)

19 February
29th ERTC Workshop - How to teach an old medicine new tricks: the importance of repurposing medicines for patients
(Brussels)

25 April
EPF General Assembly
(Online)

13 May
EURORDIS General Assembly
(Online)

14-15 May
European Conference on Rare Diseases (ECRD)
(Online)

11-14 June
European Haematology Association (EHA) Congresss

14-19 June
WFH Virtual Summit
(Online)

12 – 14 July
ISTH Virtual Congress
(Online)

27-29 July
EASL International Liver Congress
The EHC attended the Congress and held a virtual booth
(Online)

24-27 August
World Orphan Drug Congress
(Online)

28-30 October
Global Plasma Summit
(Online)

9-11 November
Reykjavík Global Forum

2 June & 16 November
EMA Patients and Consumers Working Party (PCWP) and the Healthcare Professionals Working Party (HCPWP) meetings
(Online)

THANK YOU!

VOLUNTEERS

The work of the EHC would not be possible without the enormous support from our volunteers, all those mentioned above and many, many others. We are endlessly grateful to all our volunteers from the committees, advisory bodies, working groups, events' speakers and other volunteers for their energy, time, ideas, commitment and drive to support the work of the EHC!

Rezan Abdul-Kadir, Aizat Aidarbekova, Terkel Andersen, Enea Atroce, Greta Baronaitė, Manuel Barslaag, Angelika Batorova, Jacob Bech Andersen, Jan Blatny, Valentin Brabete, Marion Brauer, Christina Burgess, Jose Caetona Martins, Pratima Chowdary, Panagiotis Christoforou, Miranda Cole, Yannick Collé, Massimo Colombo, Miguel Crato, Josh Crombie, Roseline D'Oiron, Piet de Kleijn, Manon Degenaar-Dujardin, Milica Dimic, Alison Dougall, Cristian Dragusin, Mariette Driessens, Gaetan Duport, Jo Eerens, Els Eersens, Carmen Escuriola-Ettingausen, Albert Farruggia, Enrico Ferri-Grazzi, Karin Fijn van Draat, Sara Garcia Barcenilla, Paul Giangrande, Nicolas Giraud, Jack Grehan, Evelyn Grimberg, Dan Hart, Steffen Hartwig, Marie-Laure Hecquet, Cedric Hermans, Anneliese Hilger, Vince Jenkins, Mirko Jokic, Radoslav Kaczmarek, Peter Kampman, Matej Kandare, Marie Katzerová, Marianna Kladi, Robert Klamroth, Meri Korpilahti, Michelle Lavin, Yann Le Cam, Frank Leebeek, Diana Lighezan, Sebatien Lobet, Nuno Lopes, Eric Lux, Mike Makris, Mihaela Iona Malita, Maria Elisa Mancuso, Evelien Mauser-Bunschoten, Giuseppe Mazza, Donal Mc Cann, Mark Mc Gowan, Paul McLaughlin, Wolfgang Miesbach, Linda Myrin Westesson, Lochana Nanayakkara, Beatrice Nolan, Declan Noone, Cristina Novembrino, Niamh O'Connell, Nathan O'Hagan Doyle, Jamie O'Hara, Jim O'Leary, Monica O'Leary, Brian O'Mahony, Johannes Oldenburg, David Page, Ana Pastor, Luke Pembroke, Teresa Pereira, Flora Peyvandi, Glenn Pierce, Geneviève Piétu, Steve Pipe, Debra Pollard, Stefan Radovanovic, Julia Rauscher, Olivia Romero-Lux, Frits Rosendaal, Nathalie Roussel, Thomas Sannié, Thomas Schindl, Uwe Schlenkrich, Uwe Schlenkrich, Susie Shapiro, Glenda Silvester, Mark Skinner, Naja Skouw-Rasmussen, Clive Smith, Luigi Solimeno, John Stack, David Stephensen, Malin Svennefalk, Anna Tollwe, Joanne Traunter, Nanda Uitslager, Michael van der Linde, Minette van der Ven, Karin Van Galen, Joyce Van Steenbergen, Timon van Steenbergen, Thierry Vanden Driessche, Caroline Voltz, Mark Ward, Thynn Thynn Yee, Yuri Zhylev, Baiba Ziemele, Ligita Ziemele, Janis Ziemelis.

MEPS

EHC is grateful to the Members of **the European Parliament (MEP) Rare Bleeding Disorders Group** that supports the work of the EHC, especially in the organisation of the Round Tables of Stakeholder in the European Parliament:

- **MEP Dr Cristian-Silviu Bușoi** | European People's Party (EPP), Romania
- **MEP Dr Biljana Borzan** | Socialists and Democrats (S&D) Vice-President, Croatia
- **MEP Sirpa Pietikäinen** | European People's Party (EPP), Finland
- and to **MEPs Katalin Cseh, Kateřina Konečná, Manuel Pizarro, Stelios Kympouropoulos** and **Alex Agius Saliba** for the demonstrated support for the rare bleeding disorders community.

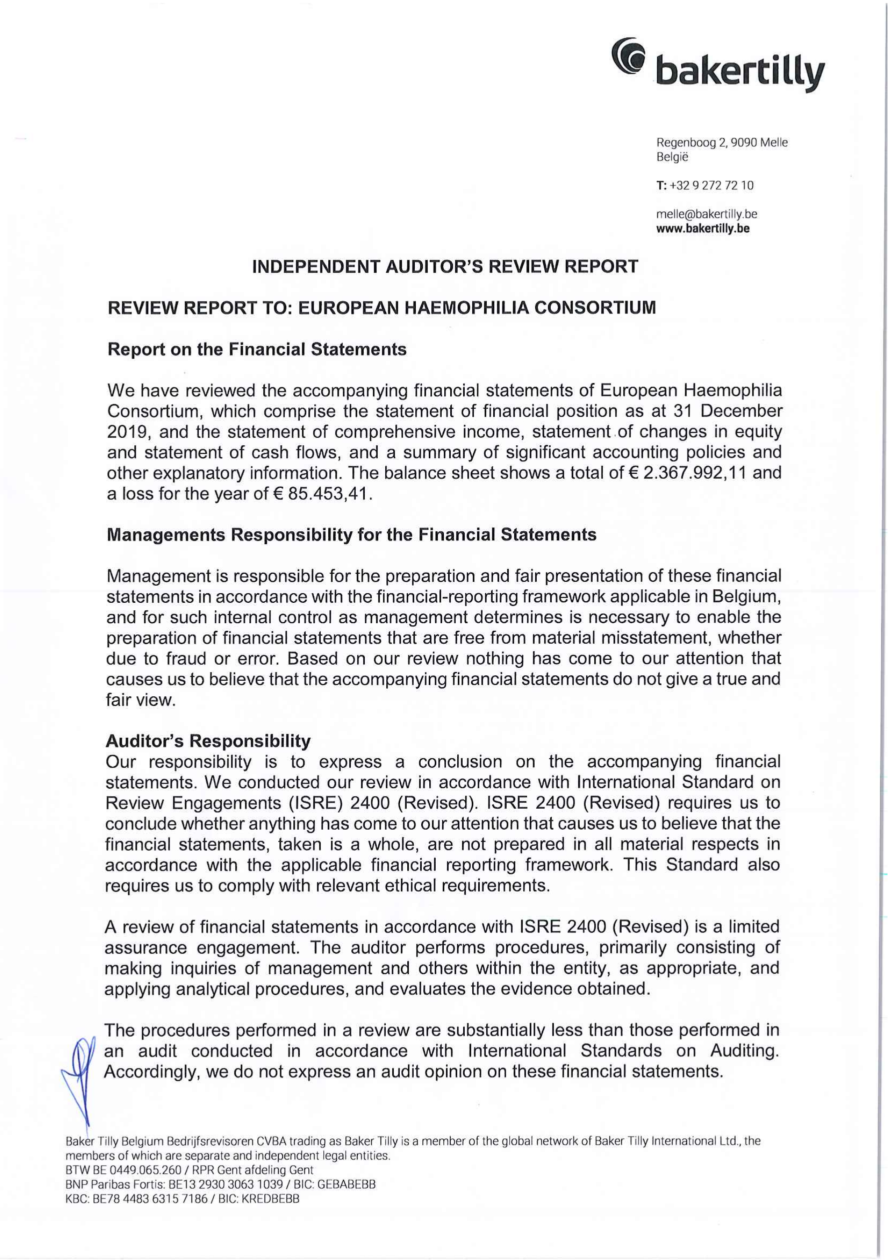
SERVICE PROVIDERS

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

- Conference Organisers
- RPP Group
- Ethical Properties
- Design-Plus
- Yaska
- Voilà
- Advantage Point
- Barretstown
- Goran Kapetanovic
- Joseph Carr
- Elliott Laub
- Jens Steen Larsen from Larsen & Co
- CitrusSuite
- Art of e-Learning
- Olga Miniuk
- Julia Krivkina
- Gabi Witthaus
- Brenda Cecilia Padilla Rodríguez
- Jeff Stanford
- Fiona Robinson
- Heather Mason
- Matt Evans

Thank you for supporting the European bleeding disorder community!

The EHC is also particularly grateful for the fully pro-bono legal services and support provided by Covington and MURAL.



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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 2019, and the statement of comprehensive income, statement of changes in equity and statement of cash flows, and a summary of significant accounting policies and other explanatory information. The balance sheet shows a total of € 2.367.992,11 and a loss for the year of € 85.453,41.

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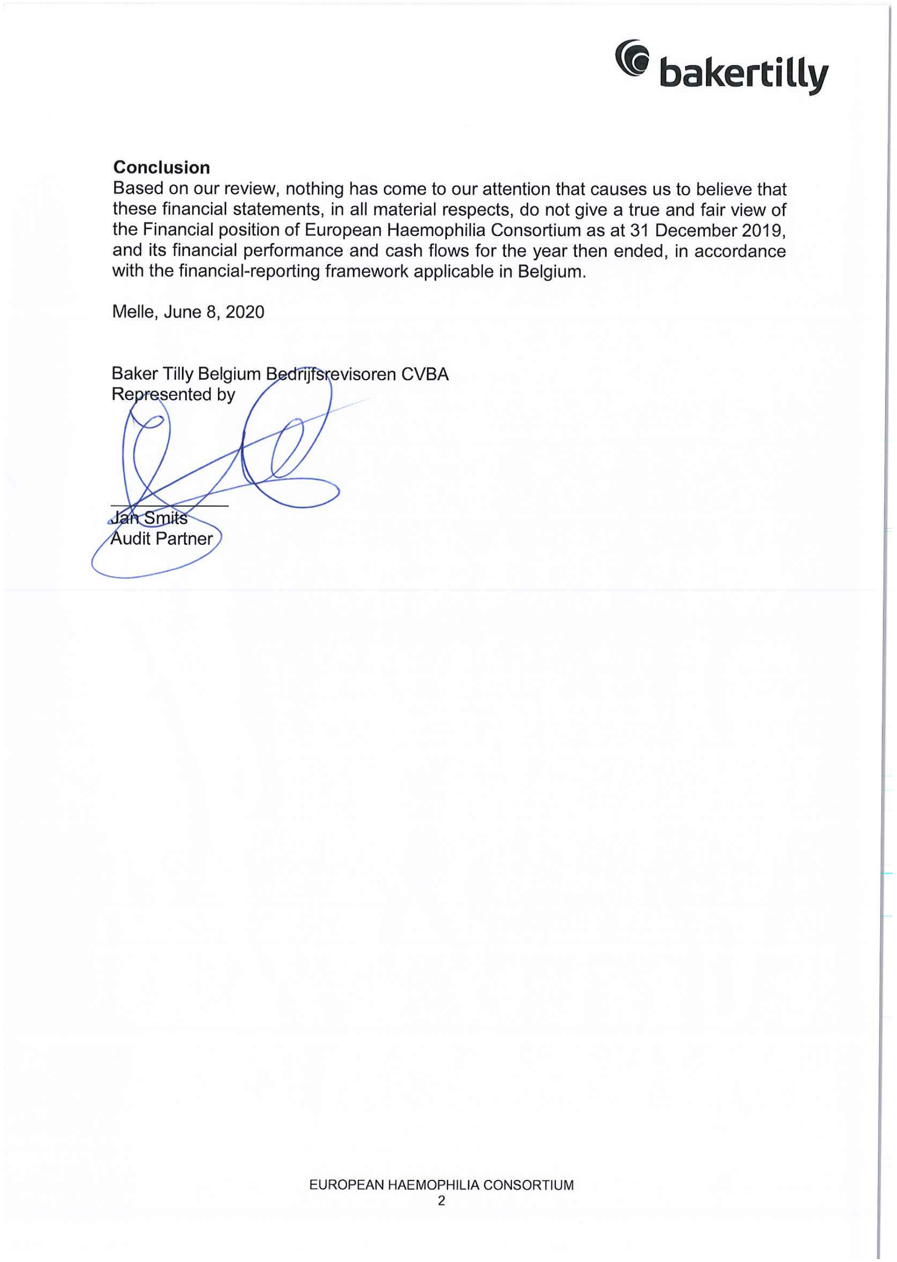

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Conclusion

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 2019, and its financial performance and cash flows for the year then ended, in accordance with the financial-reporting framework applicable in Belgium.

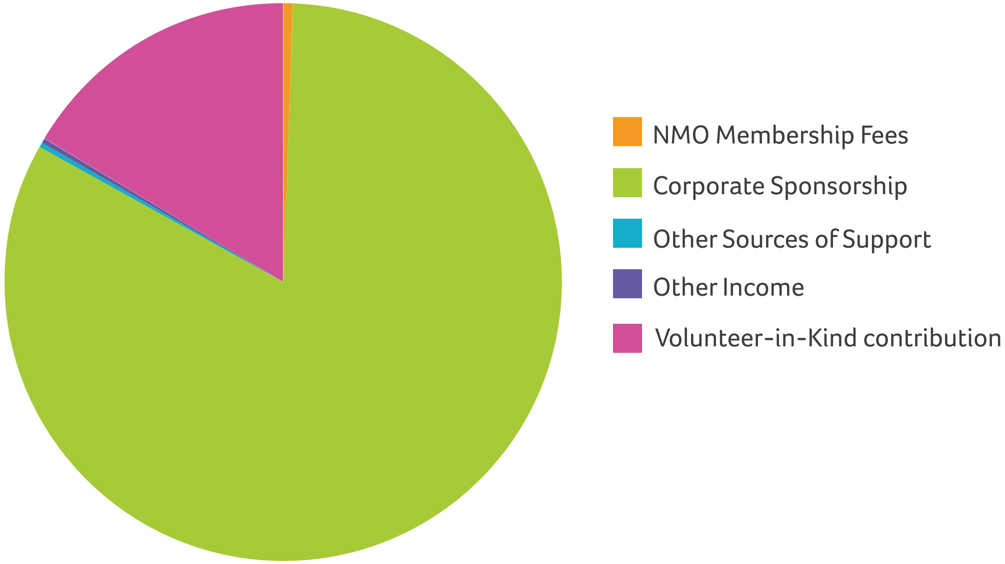
Melle, June 8, 2020

Baker Tilly Belgium Bedrijfsrevisoren CVBA
Represented by

Jan Smits
Audit Partner

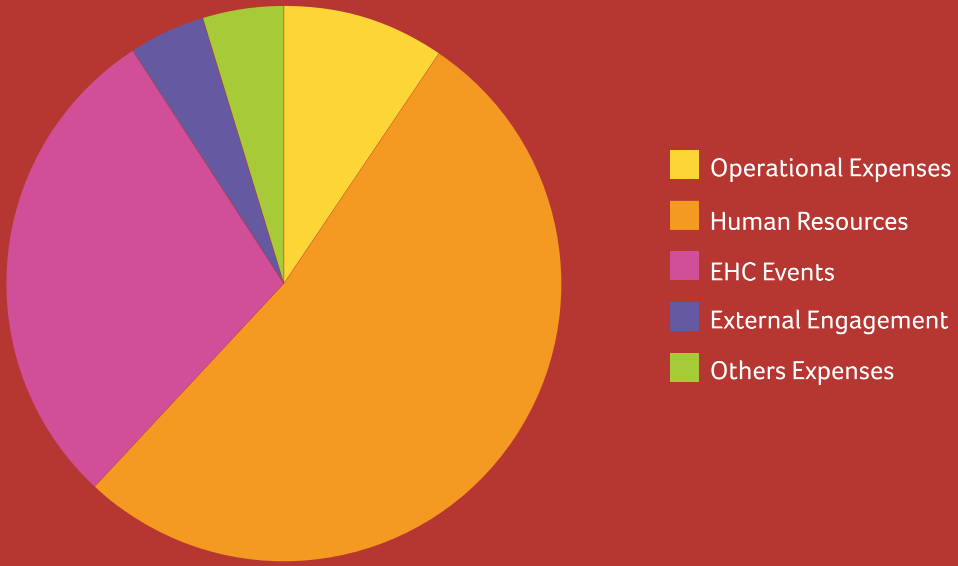
EUROPEAN HAEMOPHILIA CONSORTIUM
2

INCOME



INCOME	**FINALS (€)**
NMO Membership Fees	11,150
Corporate Sponsorship	1,581,630
Other Sources of Support	1,250
Other Income	7,748
TOTAL INCOME	**1,601,778**
Volunteer-in-Kind contribution	315,000

EXPENDITURE



EXPENDITURE	**FINALS (€)**
Operational Expenses	101,740
Human Resources	565,032
EHC Events	308,342
External Engagement	49,805
Others Expenses	48,362
TOTAL EXPENDITURE	1,073,281

BALANCE SHEET 2021
AUDITED

IT & Furniture	6.645
New Offices	534.934
FIXED ASSETS	**541.578**
Amounts Receivable	71.820
Bank Accounts	2.014.812
VAT Current Account	13.806
FINANCIAL ASSETS	**2.100.438**
Charges related to 2020	57.857
DEFERRED CHARGES	**57.857**
TOTAL ASSETS	**2.699.873**

Patrimony 2019	1.724.705
Balance 2020	528.497
EQUITY	**2.253.202**
Amounts to Pay	9.961
Taxes	10.192
Social Debts (Salary)	36.318
DEBTS	**56.472**
Income related to 2021	390.200
DEFERRED INCOME	**390.200**
TOTAL LIABILITIES	**2.699.873**

EHC 2020
CORPORATE GIVING PARTNERS

The EHC wishes to acknowledge and thank its 2020 corporate giving partners for making its work possible.

PLATINUM SPONSORS:

BioMarin | CSL | NovoNordisk | Pfizer | Roche | Sobi | Takeda

GOLD SPONSORS:

Bayer | Sanofi | UniQure

SILVER SPONSORS:

Biotest | Freelin | Sigilon

The EHC also wishes to thank the following companies for supporting individual 2020 activities and projects:

Sobi kindly sponsored the Youth Leadership Workshop, while BioMarin, Sanofi and Sobi supported the Youth Fellowship Programme. The Virtual World Haemophilia Week was made possible thanks to the support of Sobi.

The Leadership Conference was kindly sponsored by Pfizer, Roche, Sobi and Takeda, meanwhile CSL Behring, Novo Nordisk, Takeda and Bayer contributed to the success of the webinar series for Women with Bleeding Disorders.

Support from Takeda, Roche, Biomarin, Spark and Pfizer made the EHCucate educational app project come to fruition.

BioMarin and Sobi kindly sponsored the EHC virtual Economics Workshop, while BioMarin, Bayer and Sobi enabled the online Workshop on New Technologies in Haemophilia Care to happen.

Kedrion, Roche and Sobi supported the EHC PARTNERS Programme.

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

The Round Tables of Stakeholders were kindly supported by Bayer, Biotest, BioMarin, CSL Behring, Freeline, Novo Nordisk, Pfizer, Roche, Sanofi, Sobi, Spark and Takeda.

The VWD project could be launched with the support of CSL Behring and Takeda and the life of our website in 2020 was supported by BioMarin.

Finally the EHC 2020 Conference received support from BioMarin, CSL Beehring, Kedrion, Novo Nordisk, Pfizer, Roche, Sanofi, Sobi, Spark, Takeda and UniQure.

Created for patients, by patients.

CARL LYONS
Carl lives in Denmark
and has Haemophilia A

Welcome to Changing Haemophilia® >

Changing Haemophilia® was developed by Novo Nordisk to help address the unmet needs in haemophilia and other rare blood disorders. Together with you and our community partners we are striving for better access to care, diagnosis and multidisciplinary care. Here we aim to provide all people living with rare blood disorders a place that they can come to find support and advice, from us and from others.

Most read

1 min. read

World Haemophilia Day - moving forward with optimism and resilience

Optimism and resilience in unpredictable times have characterised much of the last

Introducing the all new Changing Haemophilia® website.

Where people living with haemophilia can share their stories and discover a community.
Dedicated to providing vital help and support, as well as useful tools and information.

Visit **changinghaemophilia.com**

changing haemophilia®

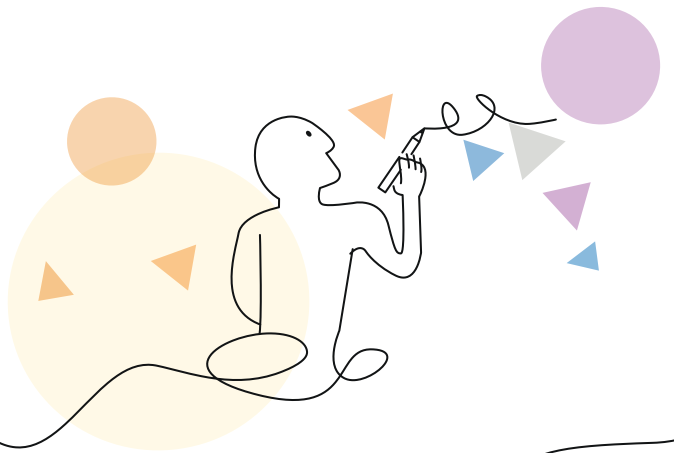
Changing Haemophilia® is a registered trademark owned by Novo Nordisk Health Care AG and the Apis bull logo is a registered trademark of Novo Nordisk A/S.
HQ21CH00014 Date of preparation: June 2021.

novo nordisk®

Addressing unmet needs

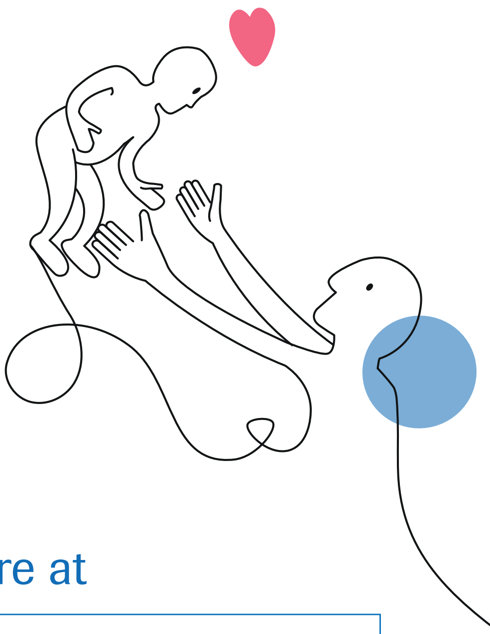
beyond bleeds

Roche



Roche's vision is to serve the needs of **every single person living with Haemophilia A**

… we **partner with the community** to challenge ourselves…



… to deliver **meaningful change that lasts for everyone around the world**

Find out more at

www.roche.com/haemophilia

Veeva Number: M-XX-00005640
Date of Preparation: June 2021

Driven by **Our Patients**

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

CSL Behring

Pfizer has driven innovation in haemophilia for over 20 years.

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

✓ develop educational patient resources & programs

✓ amplify patient voices

✓ support the haemophilia community

Pfizer has supported the following EHC-organised activities*:

EHC conferences

Round tables

Leadership conferences

Educational app

We're working to unlock the potential of gene therapy for people living with haemophilia and rare genetic diseases worldwide.

REFERENCE: Pfizer. Rare Disease Pipeline. Accessed August 12, 2021. https://www.pfizer.com/science/rare-diseases/research

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

Date of preparation: August 2021
Job code: PP-HEM-BEL-0023
210896 August 2021

To learn more about our breakthroughs in gene therapy, please visit: **www.pfizer.com/science/research-development/gene-therapy**

amplify

A Takeda-sponsored podcast series

Baiba Ziemele
President of the Latvia Hemophilia Society and the Latvian Alliance of Rare Diseases; VWD patient; and member of the EHC VWD Working Group, Latvia

Donal McCann
Executive board member of the Irish Haemophilia Society and member of the EHC VWD Working Group

Clive Smith
Hemophilia patient representative and chair of the board of The Haemophilia Society

Amplifying the voices of people living with rare bleeding disorders across Europe

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

Over the last year, we have continued to support the great work of the European Hemophilia Consortium (EHC) through various initiatives, including: Corporate Giving Program, Roundtables, European Inhibitor Network, Von Willebrand Disease Network, Women and Bleeding Disorders Conference, Annual Conference and Symposium, Leadership Conference and Patient Educational App.

We look forward to our ongoing collaboration. Through multi-stakeholder engagements we believe we can truly drive positive impact for people living with rare bleeding disorders across Europe to ensure better health and a brighter future for these communities.

Click below to listen/read more

Listen to Amplify, a podcast series for the rare bleeding disorders community.

Click here for a guide to support you and your healthcare team to make decisions about your hemophilia care and treatment.

Visit our website and find out more about our work within rare diseases.

August 2021 | C-ANPROM/INT/HG/0069

Takeda

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EHC_Haemophilia European Haemophilia Consortium