2017
ANNUAL REPORT

EUROPEAN HAEMOPHILIA CONSORTIUM

EHC
european haemophilia consortium

ACRONYMS

ATMP: Advanced Therapeutic Medicinal Products
CEO: Chief Executive Officer
CoE: Council of Europe
DAA: direct-acting antivirals
EAHAD: European Association for Haemophilia and Allied Disorders
EASL: European Association for the Study of the Liver
EC: European Commission
EDQM: European Directorate for the Quality of Medicines and Healthcare
EHC: European Haemophilia Consortium
EHL: extended half-life
ELPA: European Liver Patients Association
EMA: European Medicines Agency
ePAG: European Patient Advocacy Group
EPF: European Patients' Forum
ERN: European Reference Network
EU: European Union
HCV: hepatitis C virus
IU: international units
IWG: Inhibitor Working Group
MAG: Medical Advisory Group
MASAG: Medical and Scientific Advisory Group
MEP: Member of the European Parliament
MoU: Memorandum of Understanding
NMO: National Member Organisation
PLUS: Platform of Plasma Users
PUP: previously untreated patient
PWH: people with haemophilia
PWI: people with inhibitor
SC: Steering Committee
ToR: Terms of Reference
UNDP: United Nations Development Programme
VWD: von Willebrand Disease
VWF: von Willebrand Factor
WFH: World Federation of Hemophilia

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european haemophilia consortium

About the European Haemophilia Consortium (EHC)

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

Mission Statement

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 policies
- Monitor the status of haemophilia care in member countries
- Stimulate research in all fields related to haemophilia and other rare bleeding disorders

2017 European Inhibitor Summit

Members

The EHC has and represents 45 National Member Organisations (NMOs) from 27 European Union (EU) Member States, including all acceding countries, as well as most of the Council of Europe (CoE) Member States.

Albania
Shoqatës Shqiptare
të Hemofilikëve

Armenia
Armenian Association
of Hemophiliacs

Austria
Österreichischen Hämophilie
Gesellschaft (ÖHG)
www.bluter.at

Azerbaijan
Hemofiliyalı Xəstələrin Respublika Assosiasiyası
www.hemophilia.az

Cyprus
Παγκύπρια Οργάνωση
Αιμορροφιλικών (Π.Ο.Α.)
www.poh.org.cy

Czech Republic
Český svaz hemofiliků
www.hemofilici.cz

Denmark
Bløderforening
www.bloderforeningen.dk

Estonia
Eesti Hemofiliauhing
www.hemofiilia.ee

Hungary
Magyar Hemofília Egyesület
www.mhe.hu

Iceland
Blæðarafélag Íslands
www.hemophilia.is

Ireland
Irish Haemophilia Society
www.haemophilia.ie

Israel
היליפומה ילוחל התומע – הלע
www.hemophilia.org.il

Macedonia
Hemolog
www.hemolog.org

Moldava
Ассоциации Гемофилии
Молдовы
www.hemophilia.ucoz.ru

Montenegro
Montenegrin Association
for Hemophilia

Netherlands
Nederlandse Vereniging van
Hemofilie-Patiënten (NVHP)
www.nvhp.nl

Serbia
Udruženje hemofiličara Srbije
www.hemofilija.org.rs

Slovak Republic
Slovenské hemofilické
združenie
www.shz.sk

Slovenia
Društvo hemofilikov Slovenije
www.drustvo-hemofilikov.si/

Spain
Federación Española
de Hemofilia (FEDHEMO)
www.hemofilia.com

▷ **Belarus**
Белорусская Ассоциация
Больных Гемофилией
www.belabg.org

▷ **Belgium**
AHVH – Hemofilievereniging –
Association de l'Hémophilie
www.ahvh.be

▷ **Bosnia and Herzegovina**
Udruženje Hemofiličara
Bosne i Hercegovine
www.uhbh.org.ba

▷ **Bulgaria**
Българска Асоциация
по Хемофилия
www.hemo-bg.org

▷ **Croatia**
Društvo hemofiličara Hrvatske
www.dhh.hr

▷ **Finland**
Suomen Hemofiliayhdistys
(SHY)
www.hemofilia.fi

▷ **France**
Association Française
des Hémophiles (AFH)
www.afh.asso.fr

▷ **Georgia**
Georgian Association
of Hemophilia and Donorship
www.gahd.org.ge

▷ **Germany**
Deutsche Hämophilie
Gesellschaft
www.dhg.de

▷ **Greece**
Σύλλογος Προστασίας
Ελλήνων Αιμορροφιλικών
(ΣΠΕΑ)
http://hemophiliasociety.gr

▷ **Italy**
Federazione delle Associazioni
Emofilici (Fedemo)
www.fedemo.it

▷ **Kyrgyz Republic**
Kyrgyz Haemophilia Society
"Community of handicapped –
haemophiliacs"
http://hemophilia.go.kg

▷ **Latvia**
Latvijas Hemofilija Biedrība
www.hemofilija.lv

▷ **Lithuania**
Lietuvos Hemofiljos Associja
www.hemofilija.lt

▷ **Luxembourg**
Association Luxembourgeoise
des Hémophiles
www.alh.lu

▷ **Norway**
Foreningen for blødere
i Norge (FBIN)
www.fbin.no

▷ **Poland**
Polskie Stowarzyszenie
Chorych na Hemofilię
www.hemofilia.of.pl

▷ **Portugal**
APH – Associação Portuguesa
de Hemofilia e de outras Coagulopatias Congénitas
www.aphemofilia.pt

▷ **Romania**
Asociația Română de Hemofilie
(ARH)
www.hemofilic.ro

▷ **Russian Federation**
Всероссийского общества
гемофилии
www.hemophilia.ru

▷ **Sweden**
Förbundet Blödarsjuka
i Sverige
www.fbis.se

▷ **Switzerland**
Schweizerische
Hämophilie-Gesellschaft
www.shg.ch

▷ **Turkey**
Türkiye Hemofili Derneği
www.turkhemoder.org

▷ **Ukraine**
Всеукраїнське товариство
Гемофілії
www.hemophilia.org.ua

▷ **United Kingdom**
The Haemophilia Society
www.haemophilia.org.uk

Message from the President and Chief Executive Officer (CEO)

Brian O'Mahony & Amanda Bok

2017 was a year of closings and new beginnings.

Four years ago we set out our 2014-2017 Strategic Plan. Coming to its end in 2017, it was time to close one chapter and draft the next one. In 2013-2014, the EHC was a very different organisation and our Steering Committee (SC) and Medical Advisory Group (MAG) jointly elaborated the 2014-2017 Strategic Plan with input from public policy advisors. In 2017, the 'ownership' of the EHC looked very different, clearly extending beyond individual elected and appointed positions, into the collective of our community. For this reason we engaged a neutral and independent third-party consultant to lead a six-month external stakeholder consultation process designed to solicit feedback on the past and future directions of the organisation. The consultation process ran from August through December 2017 (and into January 2018) and provided a platform for anonymous feedback for all EHC members as well as a number of other key stakeholders, including health care providers, policy-makers, regulators, other patient organisations and industry partners, allowing them to co-determine with us the future direction of our organisation. The feedback forms the basis of the 2018-2022 Strategic Plan.

On a policy level, the valuable work of the European Commission Expert Group on Rare Diseases came to an end, having finished its mandate and following a decision by the European Commission to be discontinued. For the EHC, however, this platform was replaced by the official establishment of the rare haematological European Reference Network, EuroBloodNet, in which the EHC is formally involved both through the European Patient Advocacy Group (ePAG) and through its clinicians on all organizational levels (for more on this please see p. 41).

On an advisory level, exciting new chapters were written for women in our community, with the Women and Bleeding Disorders (WBD) Working Group leading a Women and Bleeding Disorders survey and growing into a Committee at the end of the year (see p. 49). Our Data and Economics Committee (DEC) ended its mandate, but we brought its members on to other new bodies. The most notable of these was the establishment of our Medical and Scientific Advisory Group (MASAG), which was created to provide the EHC with high-level expertise and advice from the comprehensive sectors of nursing, dentistry, physiotherapy, paediatrics, laboratory expertise, gynaecology, psychology, epidemiology, regulatory affairs, health economics, genetics, orthopaedic surgery, hepatology, blood safety, immunology and expertise in women and bleeding disorders. It held its first meeting at the EHC Conference in Vilnius (for more on this please see p. 42). We take this opportunity to thank all members of the outgoing WBD Working Group and the DEC for their invaluable support during their mandates and look forward to working together with them in new capacities!

In terms of new capacities, in 2017 the EHC also welcomed two new colleagues: a new Communications Officer - having de-linked communications from public policy work - and a PARTNERS program consultant (see p. 36). Additionally the office team moved into a larger space in the same building to accommodate its growing numbers.

Finally and perhaps most historically, 2017 saw the rolling out of the PARTNERS programme and was a busy first year with: NMO workshops; signing of consensus statements by industry partners Kedrion, Pfizer and Sobi; numerous meetings with European institutions, legal teams, national Ministers of Health, NMOs and treatment centres in the PARTNERS countries; establishment of the PARTNERS Advisory Board, programme endorsement by MEPs, EPF and EURORDIS; and the programme's official launch in the European Parliament in November (for more on this please see p. 36).

We are proud of our 2017 achievements but prouder still of the ever-growing vibrancy, energy and active engagement by ever-more members of our European bleeding disorders community. Each year we hear more voices from our community - from our youth leaders, from our patients with inhibitors and their caregivers, from our women living with or alongside bleeding disorders, from our Russian speakers - and see more of your faces at our events! We're excited to write these chapters with you, and invite you to read them in more detail on the next pages.

Brian o Mahony

Brian O'Mahony
President

Amanda Bok
CEO

EHC 2017 activities at a glance

HIGHLIGHTS FROM THE YEAR:

- Organised World Haemophilia Day event to call for the implementation of the European Directorate for the Quality of Medicines (EDQM) recommendations on haemophilia treatment and care throughout Europe
- Launched the EHC PARTNERS programme on increasing access to treatment in 14 countries, including signing a Consensus Statement with three pharmaceutical companies supporting the programme
- Established the EHC Medical and Scientific Advisory Group (MASAG)
- Created a Women's Working Group to identify action points that address challenges faced by women with bleeding disorders
- Formalised the Members of the European Parliament (MEP) Group on Rare Bleeding Disorders
- Added two new staff members to the EHC team

MEDICAL AND SCIENTIFIC UPDATES ON:

- Clinical trials in haemophilia
- New technologies in haemophilia care
- Extended half-life products and non-substitutional therapies
- Gene therapy
- Inhibitors in haemophilia

MEDICAL AND SCIENTIFIC UPDATES ON:

- Tenders and Procurement – patient and clinician involvement
- Better inclusion and addressing of the needs of people with haemophilia and inhibitors within NMOs
- NMO management, strategic planning and funding
- Communication skills and engagement of young volunteers
- NMO initiatives

DATA COLLECTION ON:

- Women and bleeding disorders – challenges in diagnosis, choice of and access to treatment, their clinical outcomes and what their unmet needs are

FEBRUARY

Initial PARTNERS programme meeting with eligible NMOs

MARCH

Round Table on 'Clinical Trials in Haemophilia'

APRIL

Youth Leadership Workshop;
World Haemophilia Day event;
Signing of Consensus Statement with three pharmaceutical companies in support of the PARTNERS programme

JUNE

Round Table on 'Orthopaedic Aspects in Haemophilia Care';
Leadership Conference

SEPTEMBER

Tenders and Procurement Workshop

OCTOBER

European Inhibitor Network (EIN) Pilot Workshop;
General Assembly;
EHC Conference

NOVEMBER

New Technologies in Haemophilia Workshop;
Round Table on 'Usage and measurement of extended half-life (EHL) coagulation factor concentrates and non-substitutional therapies';
Official launch of the PARTNERS programme in the European Parliament;
Formalisation of the MEP Group on Rare Bleeding Disorders

DECEMBER

European Inhibitor Summit


European Haemophilia Consortium
COLOURING PENCILS!

Activity Report

In 2017, the EHC continued to carry out activities in line with its 2014-2017 Strategic Plan objectives, which aim to:

1. Support and empower NMOs,
2. Promote access to optimal treatment and comprehensive care for people with congenital bleeding disorders in Europe,
3. Ensure constructive engagement with key stakeholders,
4. Increase its influence on the European policy-making environment,
5. Ensure the good governance and sustainability of the organisation.

These strategic objectives maintain the spirit of the original EHC mission while also taking into account the changing health care environment in which the EHC operates.

SUPPORT AND EMPOWER NMOs

Although the activities of the EHC vary in aim, target groups and approach, at the core of our work is the same objective: to support and empower our National Member Organisations (NMOs). Our annual programme builds on and provides targeted trainings; education on health policies, legislative and scientific developments; best practice exchanges and advocacy tools; empowering NMOs to strengthen their work nationally; and collectively addressing challenges on a European level.

To assess individual country needs and areas for improvement, the EHC also carries out yearly surveys that collect data on different aspects of haemophilia and other rare bleeding disorders treatment and care. The results are used to develop advocacy materials and have meaningfully contributed to recommendations by the European Directorate for the Quality of Medicines (EDQM) on haemophilia care, the latest set of which were adopted as a Resolution by the Council of Europe (see p. 41).

Leadership Conference

High-quality, timely communication

Internal and external communication is key to the work of the EHC. We strive to develop strong dialogue with members, stakeholders and policy-makers and inform them of developments through various media channels.

WEBSITE

- Presents information on the EHC, our events and initiatives
- Equipped with a library of information on rare bleeding disorders, treatment options and updates, health care policies and more
- Regularly updated with news and key developments in the field of rare bleeding disorders
- Over 32,000 unique visits in 2017

EHC INHIBITOR WEBSITE

- Exclusively dedicated to people with haemophilia and inhibitors (PWI)
- Contains information on EHC events and initiatives aimed at PWI
- Holds a library of information on inhibitors, treatment options and updates, exchange of experience between patients, caregivers and NMOs
- Offers a chat room for patients and caregivers so that they can connect and communicate on an international level
- Has an 'Ask the Expert' section that allows for patients and caregivers to ask any question in regards to inhibitors, which are in turn answered by relevant health care professionals

QUARTERLY HEALTH POLICY REPORT

- Published four times a year
- Available to NMOs only
- Covers relevant EU and national policy and legislative updates in the area of health care

FACEBOOK PAGE

- Used for rapid dissemination of EHC news, event updates, coverage and pictures
- Posts that highlight NMO activities and news
- Sharing of any news relevant to the rare bleeding disorders community
- Links to useful articles that deal with the daily challenges of rare bleeding disorders

TWITTER ACCOUNT

- Created to have a further reach to patients, policy-makers, health care professionals and other stakeholders
- Live updates and highlights of EHC and other major events
- Allows for increased networking between organisations, institutions and other stakeholders
- Following and re-posting of stakeholder news
- Creation and popularising of awareness campaigns

NEWSLETTER

- Published three times a year
- Features up-to-date EHC news, articles from our NMOs, interviews with health care professionals, scientific and medical developments

EVENTS REPORTS

- Produced following Round Tables to report in detail on the presentations, discussions and outcomes of the event

YOUTUBE CHANNEL

- Used to disseminate advocacy videos, EHC Conference scientific session webcasts and EHC documentaries aimed at presenting the reality of people with haemophilia (and inhibitors)

Education and training

Just as the treatment of rare bleeding disorders requires a comprehensive care approach, effectively addressing challenges of the community requires collaboration between all stakeholders involved.
The following EHC events brought together patients, health care providers, policy makers and where appropriate, representatives of pharmaceutical companies to ensure education, discussions and debates that are especially vital in the age of novel therapies.
Furthermore, many of the events were tailored to different groups within the community (youth, people with inhibitors, women with bleeding disorders, etc.) in order to better identify and concentrate on their specific needs.
We encouraged participation in events by providing travel and accommodation grants, including to our Brussels-based Round Tables, the EHC Conference and World Haemophilia Day events.

ROUND TABLE ON CLINICAL TRIALS IN HAEMOPHILIA

March 2017 – Brussels, Belgium

The development of novel haemophilia therapies is the first step in a long process of testing, observation and assessment. Before they are licensed for patients' use, it is of crucial importance to generate enough data on their safety and efficacy through clinical trials, as the results allow regulators to determine if a medicinal product can be released on the market. Given the arrival of multiple novel therapies with completely new modes of action, it is important to examine these new clinical trials and to discuss the type of data each trial is collecting.

For this reason the EHC held a Round Table on 'Clinical Trials in Haemophilia.' Taking place in the European Parliament, the event was co-hosted by Members of the European Parliament Mrs Norica Nicolai (Romania/ALDE) and Dr Miroslav Mikolášik (Slovakia/EPP) and brought together more than 50 participants.

Presentations and discussions focused on:

- Collection of epidemiological data in haemophilia
- Long-term observation of efficacy for novel therapies in haemophilia
- Safety of novel therapies
- Reporting of adverse events
- Previously untreated patients (PUPs) in clinical trials

The event's main conclusions regarded the coordination of long-term post-marketing data collection, the harmonisation/standardisation of data collection and the importance of sharing such data with all haemophilia centres.

YOUTH LEADERSHIP WORKSHOP

April 2017 – Amsterdam, the Netherlands

The EHC held its fourth Youth Leadership workshop in Amsterdam in April, welcoming 16 participants from Austria, Estonia, Finland, France, Germany, Ireland, the Netherlands, Portugal, Romania, Serbia, Slovakia and Switzerland. Working off the strengths of their diversity, the workshop explored ways in which the participants could be better involved in their patient organisations while 'arming' them with the tools to pursue their ideas and a leadership role in the bleeding disorders community in the future. The programme of the event combined presentations, interactive activities and case studies that covered:

- Attracting and retaining volunteers
- Communication techniques
- The process of medicines development and marketing authorisation
- Working with stakeholders
- Leadership characteristics
- Role and building of patient organisations
- Collaboration between generations within a patient organisation

Participants were provided with opportunities to further develop their skills and ideas following the workshop. Many attended other EHC events, took part in the Youth Debate (see p. 23) or joined various EHC committees.

This workshop was made possible thanks to educational grants from Novo Nordisk, Roche and Shire.

TENDERS AND PROCUREMENT WORKSHOP

September 2017 – Sofia, Bulgaria

The EHC Tenders and Procurement Workshop is a training event that brings to the forefront a concept the organisation passionately advocates for: the involvement of haemophilia patient representatives and clinicians in the procurement of treatment products. As the results of the procurement directly impact the availability of treatment, its quality, safety and therefore, the quality of life of people with haemophilia, it is of extreme importance to integrate a collaborative approach in the decision-making process.

The 2017 Tenders and Procurement Workshop provided patient representatives from Albania, Bulgaria, Croatia, Estonia, Greece, Hungary, Israel, Lithuania, Macedonia, Poland and Serbia with the technical and practical skills to actively participate in their national tender/procurement processes. The two-day training covered all relevant concepts, from treatment evolution and safety criteria throughout the years, to the breakdown of how tender and procurement systems work throughout Europe. Participants also took part in a mock tender, during which they had to develop treatment selection criteria and score factor concentrate products. They were presented with different scenarios and conditions under which treatments were developed and had to make a decision based on which one they thought was the most safe, efficacious and cost effective, amongst other criteria.

This workshop was made possible thanks to an educational grant from Pfizer.


Extended half-life products (EHL)
Standard therapy
Plasma-derived
Recombinant
Half-life
Extended half-life products
Extension of half-life
rFVIII 1.4 – 1.6 fold
rFIX 3 – 5 fold
Frequency of infusions (2-3 time per week)
Reduction of infusions
EHC

EHC CONFERENCE

October 2017 - Vilnius, Lithuania

The EHC Conference is one of the largest patient gatherings in Europe and each year aims to provide the latest treatment information as well as be a place for exchange of experiences between NMOs and open dialogue between patients and health care experts. Held in October in Vilnius, Lithuania, the 2017 EHC Conference brought together more than 300 participants that attended a mixture of scientific lectures, debates and symposia sessions.

As preparedness is key, the event provided in-depth education to help NMOs both to be prepared for, as well as take active leadership in, addressing the issues that novel haemophilia treatments will bring, as well as to continue tackling inequalities in haemophilia care across their respective countries.

The programme consisted of:

- Overview of haemophilia treatment and care in Lithuania and the Baltics
- Multidisciplinary collaborations
- Novel products and gene therapy
- Pharmacovigilence
- Orthopaedic issues
- Rehabilitation after total hip and knee arthroplasties
- Radioactive synovectomy
- Nursing elements in haemophilia
- Inhibitor management
- Instruments in outcome measures
- Management of pregnancy in women with constitutional bleeding disorders

A highlight of the conference was once again the Youth Debate, which brings young and dynamic voices into a debate against established leaders in the haemophilia community on key, and often controversial, issues. This year, three youth representatives – Valentin Brabete (Romania), Robbert-Jan 't Hoen (Netherlands) and Tobias Krucker (Switzerland) had ten minutes to argue their positions on a previously assigned topic before their opponents took the stage.

NEW TECHNOLOGIES WORKSHOP

November 2017 – Estoril, Portugal

The future arrival of novel haemophilia therapies on the market will require a great deal of education for patients, health care providers and treatment centres in order to assure a safe and beneficial transition. The 2017 EHC New Technologies Workshop generated the largest interest to-date, reaching maximum participant capacity shortly after registration opening.

The programme covered the technologies of extended half-life factors, gene therapy and non-substitutional products in lay language and allowed participants to develop a more in-depth understanding of each of these novel therapies.

Presentations were based on the latest research and clinical trial results and provided for open discussions between patient representatives and health care professionals on the potential of each technology and its impact on treatment and care. Discussions included changing patient expectations as we strive beyond achieving survival, to achieving a high quality of life, and considerations around the potential for inhibitor development when current treatments are working well.

This workshop was made possible thanks to educational grants from Roche, Shire and Sobi.

ROUND TABLE ON 'USAGE AND MEASUREMENT OF EHL AND NON-SUBSTITUTIONAL THERAPIES'

November 2017 – Brussels, Belgium

The EHC Round Table on 'Usage and Measurements of Extend Half Life (EHL) and Non-Substitutional Therapies' aimed to explore the current landscape of use and the safety, inhibitor development and bleeding rates of EHL and non-substitutional therapies. The event was an opportunity to present participants with real-life examples of countries that have made the switch to these therapies, as well as provide information on how their safety and efficacy is monitored.

Presentations covered the following topics:

- Current use of EHL and non-substitutional therapies
- Monitoring efficacy: measuring EHL and non-substitutional therapies
- Monitoring safety: pharmacovigilance programmes for EHL and non-substitutional therapies
- Assessing value: comparative assessment of standard treatment vs novel technologies

The event ended with a comparative assessment of standard treatment vs novel technologies and a panel discussion on what haemophilia treatment might look like ten years into the future: in 2027.

EUROPEAN INHIBITOR SUMMIT

December 2017 – Barretstown, Ireland

After a successful pilot, the EHC was excited to organise the 2017 EHC Inhibitor Summit and bring together over 110 participants, speakers and facilitators from 27 European countries. Participants included adults, children, patients, partners, siblings and relevant experts, allowing the group to learn, share and continue to build a community of support for people who are often isolated due to the small number of patients on a national level.

The programme of the Summit consisted of both informational and practical workshops and included:

- Information sessions on latest advancements in inhibitor treatment, nutrition and weight monitoring, dental care, immune tolerance induction, and mindfulness
- Workshop on physiotherapy for adults and children
- Peer-to-peer sessions and home groups
- Leisure and various activities for children and families

This event was made possible thanks to educational grants from Alnylam, Roche and Shire.


EHC
european haemophilia consortium
EHC INHIBITOR SUMMIT
2017
Roche
Shire

Support and assist on national advocacy

In addition to providing information and targeted advocacy materials to its NMOs, the EHC also supports individual NMOs in their national advocacy work on an ad hoc basis and as needed.

EHC ACTIVITY GRANT

To further support NMOs in their community efforts, in 2016 the EHC created an activity grant that could be applied and used for co-funding activities that aim to improve the quality of life of people with haemophilia and other bleeding disorders. The fund can be used towards new or existing areas of work in a short-term and ad-hoc fashion until sustainable funding can be secured by the NMO.

In 2017, grants were awarded to:

- The Ukrainian NMO to organise a summer camp for children with haemophilia and their families
- The Georgian NMO to send participants to the Ukrainian NMO summer camp for children with haemophilia and families
- The Lithuania NMO to organise a summer basketball camp for young men with haemophilia from Lithuania and neighboring countries
- The Armenian NMO to send participants to the Lithuanian NMO basketball camp for young men with haemophilia
- The Danish NMO to create and publish a booklet about their work and offered services
- The Hungarian NMO to hold a camp for youth with haemophilia
- The Kyrgyz NMO to travel around the country and assess the quantity of treatment product used across the country


Lithuanian summer basketball camp

TARGETED NATIONAL ADVOCACY SUPPORT

In 2017 the EHC continued to support advocacy efforts in Greece and Ukraine, lending particularly targeted support to Ukraine's procurement process for haemophilia treatment products. The EHC signed a Memorandum of Understanding with the United Nations Development Program (UNDP) Ukraine, which carried out the procurement process for haemophilia treatment products on behalf of Ukraine's government, and provided them with active and close collaboration throughout the year.

The EHC also provided targeted advocacy support to Switzerland in their efforts to have access to hepatitis C treatment with direct acting agents for people with bleeding disorders.

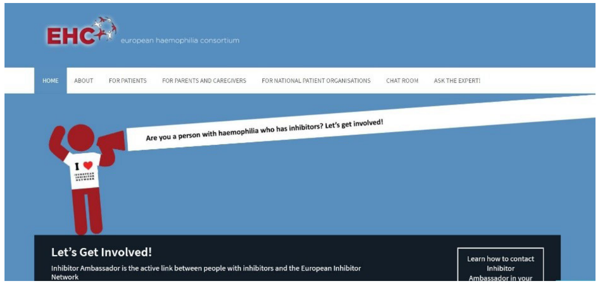
SCREENING OF THE DOCUMENTARY "INHIBITOR STORIES"

October 2017 – Vilnius, Lithuania

In 2017, the EHC once again collaborated with director and film-maker Goran Kapetanovic to produce a documentary that sheds light on the difficult issues people with haemophilia and inhibitors are often faced with. *Inhibitor Stories* captures the powerful experiences of six people with haemophilia and inhibitors from throughout Europe, highlighting the different outcomes that disparities in access to treatment lead to. The documentary was premiered at the 2017 EHC Conference (see p. 23) and is available to NMOs to use in their efforts to spread awareness and advocate for improvements in treatment and care.

LAUNCH OF THE INHIBITOR PLATFORM

As part of the work plan of the EHC European Inhibitor Network (EIN) to better support people with haemophilia and inhibitors (PWI) across Europe, in 2017 we launched an online platform specifically dedicated to that part of the community. Following a survey to accurately identify the needs of those living with an inhibitor, the EIN developed several programme elements to meet the identified needs. Providing PWI with an online platform that creates a community space for them, as well as a resource for information and educational materials, discussions and connectivity to each other was one such need.



Are you a person with haemophilia who has inhibitors? Let's get involved!

Let's Get Involved!

Inhibitor Ambassador is the active link between people with inhibitors and the European Inhibitor Network

Learn how to contact Inhibitor Ambassador in your

The resulting website was divided into sections that are tailored to patients, parents and caregivers, as well as to national patient organisations, with ideas on activities they can implement within their own inhibitor communities for better support and inclusion. Besides being used as an online library for resources on the topic, visitors also have an opportunity to communicate with their peers throughout Europe through a chatroom, as well as ask any question they have around inhibitors to health care professional from different medical fields in the 'Ask the Expert' section.

Data collection and sharing of best practices

Sound data evidence is imperative in driving change and therefore data collection is one of the EHC's core activities in order to achieve treatment and care improvements on both national and European levels. We conduct annual surveys that collect data on various aspects of access to treatment and care throughout Europe, as well as on challenges faced by different groups within the community (women with bleeding disorders, people with haemophilia and inhibitors, etc.). The analysed results are frequently published in scientific journals, such as *Haemophilia*, and have been significantly influential in providing evidence for policy recommendations at a European level, such as the recommendations for haemophilia treatment from the European Directorate for the Quality of Medicines and Healthcare (EDQM). In 2015, and again in 2017, the respective recommendations were turned into Council of Europe Resolutions (see p. 41).

The EHC also uses its events to provide opportunities for best practice exchange between NMOs, allowing for members to develop initiatives and better support their national bleeding disorders community.

LEADERSHIP CONFERENCE

June 2017 – Brussels, Belgium

The third EHC Leadership Conference, held in late June/early July, once again brought together established leaders, staff and young volunteers of various EHC National Member Organisations (NMO) for a weekend of discussions, best practice exchange, educational presentations and interactive sessions.

The different backgrounds and experiences of the participants made for a diverse group that eagerly shared ideas around the common goal of providing the best possible support to people with haemophilia and their families. In 2017 a new element of poster sessions was added to the programme, which offered an opportunity for an in-depth exchange of best practices. NMOs created poster displays of key initiatives they had organised in their respective countries, and then presented them to others. These included "Starting a conversation with people with mild haemophilia" (French NMO); "Building a strategic plan" (French NMO); "Building awareness of psychosocial aspects of haemophilia" (Serbian NMO); "Haemophilia and Ageing" (UK NMO); "Applying informed consent to haemophilia treatment" (Portuguese NMO); "Haemophilia and life as a couple" (Romanian NMO); and "Interaction of the Russian Haemophilia Society with other patients' societies" (Russian NMO).

The programme of the event included thematic sessions on:

- Gene therapy
- Women and bleeding disorders
- Extended half-life products
- Fundraising

Interactive elements of the event included peer-to-peer sessions, where participants broke out into groups and discussed the following topics:

- Engagement with industry
- Volunteer-staff collaborations
- Patient-clinician engagement
- Inter-generational dialogue
- Succession planning
- Using the EDQM recommendations (see p. 41) in national advocacy

This EHC Leadership Conference was made possible thanks to educational grants from Pfizer, Roche, Shire and Sobi.

SURVEY ON WOMEN AND BLEEDING DISORDERS

An often underserved group within the community are women with bleeding disorders, leaving their challenges and needs unaddressed. Their road to diagnosis and treatment can still be long and difficult as they struggle to find the necessary support on medical and personal levels. To help identify and meet their needs, in 2017 the EHC launched a 'Women and Bleeding Disorders' survey that was sent out to NMOs, treatment centres and individual patients. The questions were designed to elicit data on existing challenges, access to care and medical services, availability and range of treatments, impact on quality of life and clinical outcomes.

The collected data included individual responses from over 700 affected women. Findings and analysis of the study are expected to be published by the end of 2018, with the EHC already taking measures to address some of the identified daily barriers women face.

EUROPEAN INHIBITOR NETWORK PILOT WORKSHOP

Prior to the 2017 EHC Conference (see p. 23), the EHC Inhibitor Working Group (IWG) held a pilot workshop for invited NMOs members on better inclusion of people with inhibitors within the work of their patient organisations. The programme was carefully and methodically prepared to be relevant to the participating NMOs, with sessions focusing on:

- Understanding the specific needs of the participating NMOs in order to help them better include people with inhibitors nationally
- Presentation on the needs of people with inhibitors, in areas such as medical, social, psychological, educational and employment
- Examples from participating NMOs on ways they support people with inhibitors
- Tools on inclusion

On several occasions, participants were also split into smaller working groups tasked with fidning ways to address the challenges faced by people with inhibitors, which were raised in the sessions.

NMO liaison

In 2015 the EHC started allocating to each SC member (with the exception of the President and the ex-officio members such as the EHC CEO and the President of the World Federation of Hemophilia) nine NMOs so that each EHC member can have direct contacts with the EHC SC. In return, SC Members can give and receive information about current affairs of NMOs and provide an update to NMOs about SC decisions.

Jordan Nedevski *Vice-President*	**Radoslaw Kaczmarek** *SC Member*	**Naja Skouw-Rasmussen** *SC Member*	**Miguel Crato** *SC Member*	**Olivia Romero-Lux** *SC Member*
Armenia	Czech Republic	Denmark	Albania	Austria
Azerbaijan	Estonia	Finland	Bosnia-Herzegovina	Belgium
Belarus	Hungary	Iceland	Croatia	Cyprus
Bulgaria	Latvia	Ireland	Greece	France
Georgia	Lithuania	Israel	Macedonia	Germany
Kyrgyzstan	Moldova	Netherlands	Montenegro	Luxembourg
Russia	Poland	Norway	Portugal	Italy
Turkey	Romania	Sweden	Serbia	Slovenia
Ukraine	Slovakia	UK	Spain	Switzerland

PROMOTE ACCESS TO TREATMENT AND COMPREHENSIVE CARE

There is widely disparate access to optimal treatment and comprehensive care in different European countries. Even where optimal levels are achieved, they often become vulnerable to economic and political pressures. In 2017, the EHC took part in initiatives and organised several activities to meet its strategic objective of promoting access to treatment and comprehensive care. It also launched its most ambitious programme to date: the Procurement of Affordable Replacement Therapies – Network of European Relevant Stakeholders (PARTNERS) programme (see p. 36).

WORLD HAEMOPHILIA DAY

April 2017 – Strasbourg, France

The EHC uses World Haemophilia Day not only as an opportunity to bring attention to challenges faced by the haemophilia community, but also as a chance to come up with concrete solutions. In 2017 World Haemophilia Day was hosted by the European Directorate for the Quality of Medicines and Healthcare (EDQM), part of the Council of Europe, in Strasbourg, France and highlighted the latest recommendations (see p. 41) on optimal treatment of haemophilia in Europe.

During the event and in the presence of patients, health care professionals, regulators and pharmaceutical industry representatives, the EHC also presented important details on a new programme for improving access to treatment for people with bleeding disorders in Europe. The Procurement of Affordable Replacement Therapies – Network of European Relevant Stakeholders (PARTNERS) programme seeks to ensure increased and sustainable access to treatment in up to 14 European countries that do not meet the minimum standards of haemophilia care, as outlined by the 2016 Wildbad Kreuth consensus recommendations. These countries, where levels of Factor VIII and Factor IX consumption are below 4 international units (IU)/capita and 0.5IU/capita, respectively, provide little or no prophylactic treatment for children and adults with severe haemophilia.

Both programme elements have the ability to lead to real, substantial change and led to important discussions during the event on which steps should be taken to implement them in countries across Europe.

This event was made possible thanks to educational grants from Alnylam, Roche and Sobi.

ROUND TABLE ON 'ORTHOPAEDIC ASPECTS' IN HAEMOPHILIA CARE

June 2017 – Brussels, Belgium

The EHC Round Table on 'Orthopaedic Aspects in Haemophilia Care' brought together diverse health care professionals such as physiotherapists, haematologists, inhibitor specialists and orthopaedic surgeons, demonstrating the need for a comprehensive and multidisciplinary approach to the treatment of haemophilia. Speakers highlighted that haemophilia can impact affected individuals' musculoskeletal health and looked at procedures and methods to improve patients' health such as adapted physiotherapy regimens, radioactive synovectomy and the use of ultrasound technologies to identify bleeds.

Presentations covered the following topics:

- What are the goals of treatment from a haematologists' perspective
- Role of physiotherapist in preventing arthropathy
- Ultrasound imaging
- Radioactive synovectomy
- Elective orthopaedic surgery in patients with inhibitors

EHC PARTNERS PROGRAMME

After laying out its framework the previous year, in 2017 we officially launched our groundbreaking Procurement of Affordable Replacement Therapies – Network of European Relevant Stakeholders (PARTNERS) programme. Continuing our efforts to improve the quality of life of people with rare bleeding disorders, the programme aims to increase access to replacement factor therapies in European countries that provide inadequate levels of treatment to their haemophilia patients.

Disparities in access to treatment and care are still evident between many countries, the result of which is reflected in the quality of life of the affected individuals. The EHC PARTNERS programme draws on the experiences that have led to the best outcomes for patients, such as the involvement of clinicians and patients in the procurement process of treatment products.

Participating countries, where levels of factor VIII and factor IX consumption are below the recommended 4IU/capita and 0.5IU/capita respectively, include:

- Albania
- Armenia
- Azerbaijan
- Belarus
- Bosnia and Herzegovina
- Bulgaria
- Estonia
- Kyrgyz Republic
- Latvia
- Macedonia
- Romania
- Serbia
- Turkey
- Ukraine

In February, an initial meeting was held with NMO representatives and key clinicians of the above countries, each of them presenting their current system of purchasing factor therapies and the individual national challenges they face.

Following the meeting, the EHC held a number of activities that marked the major developments of the programme as they were carried out. With the involvement of pharmaceutical companies being vital for the success of PARTNERS, during our World Haemophilia Day event, three pharmaceutical companies – Kedrion Biopharma, Pfizer and Sobi – signed a Consensus Statement in support of the programme. Their commitment to PARTNERS was expressed through their willingness to bid in national and regional tenders organised in the PARTNERS countries. A more formal Memorandum of Understanding was signed by Kedrion Biopharma and Sobi at the end of the year.

Signing of the consensus statement

In November, in order to highlight and gain support on an international and political level, the EHC officially launched the PARTNERS programme in the European Parliament. Attending Members of the European Parliament (MEPs) included MEPs Norica Nicolai (Romania), Dr Cristian Bușoi (Romania), Nessa Childers (Ireland) and Wim van de Camp (Netherlands), all of whom stressed the need for such a programme and expressed willingness to help where possible.

MEP Norica Nikolai (Romania) spoke in support of the PARTNERS programme

Announcing their organisations' endorsement of the programme, Camille Bullot from the European Patients' Forum (EPF) and Yann Le Cam from EURORDIS – Rare Diseases Europe both spoke at the event, highlighting the innovative approach of PARTNERS.

Zafar Yuldoshev from the United Nations Development Programme, Yann le Cam, CEO of EURORDIS - Rare Diseases Europe and Camille Bullot from EPF

Throughout the year, EHC PARTNERS programme consultant Declan Noone visited the majority of the participating countries to assess their current situation in terms of haemophilia treatment and care and to determine the best approach for the implementation of the programme. He has held multiple meetings with national regulatory authorities, government ministers, hospital management and insurance agencies. Advancements can already be noted in many of the countries and the gained momentum in 2017 trickled over into the continued success of PARTNERS in 2018.

Declan Noone, EHC PARTNERS programme consultant (middle), during his visit to Albania to discuss the implementation of the programme

ENSURE CONSTRUCTIVE ENGAGEMENT WITH KEY STAKEHOLDERS

The EHC operates in a complex environment where many other stakeholders, such as patient organisations and professional societies, carry out parallel actions that have an impact on haemophilia, rare bleeding disorders and the EHC activities in general. The EHC works closely with key partner organisations, medical and professional societies, to ensure collaboration and alignment. This includes signing Memoranda of Understanding, collaborating on projects and position statements and attending and speaking at external events.

Patient Organisations

WORLD FEDERATION OF HEMOPHILIA (WFH)

The EHC and the WFH share the same NMOs and, as a result, work closely together. Additionally, the President of the WFH sits as ex-officio member on the EHC Steering Committee and in turn, the EHC President or his/her designee is invited annually to attend one WFH Board meeting. This is to ensure that both organisations work organically and do not duplicate activities. Both organisations stay in regular close contact, attending each other's annual conferences and coordinating joint positions at European, international and national levels when necessary and appropriate.

In 2017, EHC President Brian O'Mahony attended the WFH Global Forum, presenting the EHC PARTNERS programme and its aims to the global community.

EUROPEAN PATIENTS' FORUM (EPF)

The EHC is a member of EPF, an umbrella patient organisation representing chronic and lifelong conditions across Europe.
In 2017, the EHC continued to take active part in their work on access to health care and interfaced on EPF projects at European and national levels.

PLATFORM FOR PLASMA USERS (PLUS)

The members that form PLUS are the EHC, Alpha-1 Global, Guillain-Barré syndrome (GBS)/Chronic inflammatory demyelinating polyneuropathy (CIDP) Foundation International, Hereditary Angioedema international (HAEi), the WFH, American Platform of Plasma Users (APLUS) and the International Patient Organisation for Primary Immunodeficiency. PLUS meets every year to discuss pressing topics concerning plasma products. Mr Brian O'Mahony, EHC President, was elected to the PLUS SC in 2015.

EURORDIS - RARE DISEASES EUROPE

The EHC is a long-standing member of EURORDIS – Rare Diseases Europe, an international patient organisation representing over 700 patient groups active in the area of rare diseases. In 2017, the EHC was an active participant of EURORDIS' Rare Disease Day activities and the EURORDIS membership meeting.

Medical and professional societies

EUROPEAN ASSOCIATION FOR HAEMOPHILIA AND ALLIED DISORDERS (EAHAD)

EAHAD is an organisation representing health care professionals working in the area of haemophilia and other rare bleeding disorders. The EHC and EAHAD have a Memorandum of Undersanding, which outlines the terms of collaboration. The EHC and EAHAD speak at each other's conferences (see p. 23) and other events and have formal meetings twice a year. Additionally, they often consult each other with regard to the development of position statements, surveys and other relevant documents. In 2017 the two organisations also collaborated on establishing the European Principles of Inhibitor Management, which have now been published.

EUROPEAN ASSOCIATION FOR THE STUDY OF THE LIVER (EASL) INTERNATIONAL LIVER FOUNDATION

After holding a consultative meeting at the EHC Conference in Vilnius, the EHC and the EASL International Liver Foundation established a partnership to collaborate on eradicating hepatitis C from the haemophilia community by 2022. The leadership of the two organisations will work together to assure that all patients with haemophilia throughout Europe have access to direct acting anti-viral agents, which have proven to eliminate the virus.

INCREASE INFLUENCE ON THE POLICY-MAKING ENVIRONMENT

The European policy-making environment is a layered arena of multiple decision-making bodies and diverse experts, all of whom need to understand the specifics of both haemophilia and other bleeding disorders. The EHC is committed to reaching out and providing the necessary information to policy-makers in order to ensure that informed decisions are taken in policiy-making that affect the lives of people living with these conditions in Europe.

Formalisation of MEP Group on Rare Bleeding Disorders

In 2015, the EHC initiated the formation of an informal Members of the European Parliament (MEP) Group on Rare Bleeding Disorders as a result of politicians' will to collaborate and address the challenges faced by this community. On November 28th 2017, the group was officially formalised to give a further political thrust to their vision of improving the quality of life of people affected by these conditions.

The group is composed of Heinz Becker (EPP, Austria), Cristian-Silviu Buşoi (EPP, Romania), Nessa Childers (S&D, Ireland), Dr Miroslav Mikolášik (EPP, Slovakia), Norica Nicolai (ALDE, Romania) and Dr Paul Rübig (EPP, Austria). Members have chaired, supported and spoken at several EHC Round Tables and have gotten to know the issues of the bleeding disorders community from personal accounts and event discussions.

The group's objectives include: to further raise awareness on these topics; to take concrete actions where possible; and to support ways to achieve improvements nationally.

Council of Europe updated recommendations on haemophilia care in Europe

In mid-December, the Council of Europe's Committee of Ministers updated its recommendations on haemophilia treatment and care in Europe to include the 2016 European Directorate for the Quality of Medicines and Healthcare (EDQM) recommendations. This set of recommendations includes the prioritisation of hepatitis C treatment for people with haemophilia; access to immune tolerance induction (ITI) for all people with inhibitors; and minimum per capita consumption of 4 International Units (IU) of FVIII and 0.5 IU/capita pf FIX in every country. The updated recommendations bring together the EDQM recommendations from 2013, previously endorsed by the Council of Europe, with the 2016 EDQM recommendations. Together, they serve as a powerful tool to advocate for improvements in haemophilia care and access to treatment on national levels. The EHC played an important role in the establishment of these recommendations, both through providing patient data and by taking active part in the meetings from which the recommendations ensued.

To highlight the significance of the latest set of recommendations, the EHC also produced an advocacy video that emphasizes the impact that these recommendations can make in the quality of life of people with haemophilia, and supports their rapid national implementation.

EuroBloodNET

In December 2016 the European Commission approved the creation of 24 European Reference Networks (ERNs), which allow for health care professionals across Europe to collaborate and exchange knowledge on rare and complex diseases. Amongst them is EuroBloodNet, the ERN for rare haematological disorders, including haemophilia and other rare bleeding disorders. The ERNs became active as of the 1st March 2017.

In parallel, EURORDIS created European Patient Advocacy Groups (called ePAGs) for each ERN disease grouping to ensure the integration of patient voices in the structure, governance and work of the networks. EHC CEO Amanda Bok was selected alongside six other patient organisation representatives to join the ePAG overlooking EuroBloodNet. In this capacity, Ms Bok has spoken at various ePAG meetings and has actively contributed to the establishment of the EuroBloodNet work plan. Additionally, she gave a training on ePAG involvement at a pre-meeting that took place before the EURORDIS Membership Meeting in May.

European Directorate for the Quality of Medicines and Healthcare (EDQM)

The EDQM, a Council of Europe Directorate, is responsible for protecting public health by enabling the development, supporting the implementation and monitoring the application of quality standards for medicines and their safe use. It is one of the main partners of the Wildbad Kreuth initiatives (see p. 41) and the EHC works closely with them on the development of standards and recommendations for haemophilia treatment and care.

European Medicines Agency (EMA)

The EHC is one of the patient organisations recognised by the EMA and in 2017, EHC representatives took part in various events organised by the agency.

In addition, Dr Günter Auerswald continues to sit on the EMA Paediatric Committee as an EHC representative.

ENSURE GOOD GOVERNANCE AND SUSTAINABILITY

The EHC maintains open and transparent relations with all stakeholders, including pharmaceutical companies, payers and regulators. The EHC is an eligible patient organisation associated with the European Medicines Agency (EMA) and is also on the Transparency Register of the European Parliament. The EHC receives, amongst others, financial support from the pharmaceutical industry in accordance to a strict policy that is published on its website. All EHC staff, consultants, elected and non-elected volunteers, as well as members of the Medical Advisory Group (MAG) and the Medical and Scientific Advisory Group (MASAG), have to adhere to a code of conduct and declare conflict of interests.

The EHC continually seeks to explore opportunities to diversify its sources of funding.

Sound and transparent financial policies and procedures

As the staff and activities of the EHC grow, it must ensure sound and transparent financial management to ensure good governance and sustainability. To this end, the EHC has in place strict policies and procedures ensuring robust checks and balances between staff and volunteers on all payment approvals and transfers. The EHC works with an external accounting firm and reports cash flow to the Steering Committee on a quarterly basis. The EHC annual accounts are submitted to an external audit, which is made public and published at the end of this report and which is also submitted to key partners, such as the EMA.

Maintain neutral and transparent corporate sponsorship policy

Much of the EHC’s work is made possible by generous support from its corporate partners. In order to ensure ethical and transparent relationships, the EHC accepts corporate funding in strict compliance with its ‘Policy on Financial Support from Corporate Sponsors,’ which is published on the EHC website, and which outlines the terms and conditions relating to such funding, including that the EHC shall exercise full independence, accountability and transparency.

Adhere to clear and ethical codes of conduct

In 2017, the EHC updated its codes of conduct in order to align it with the organisation’s newly established activities and responsibilities.

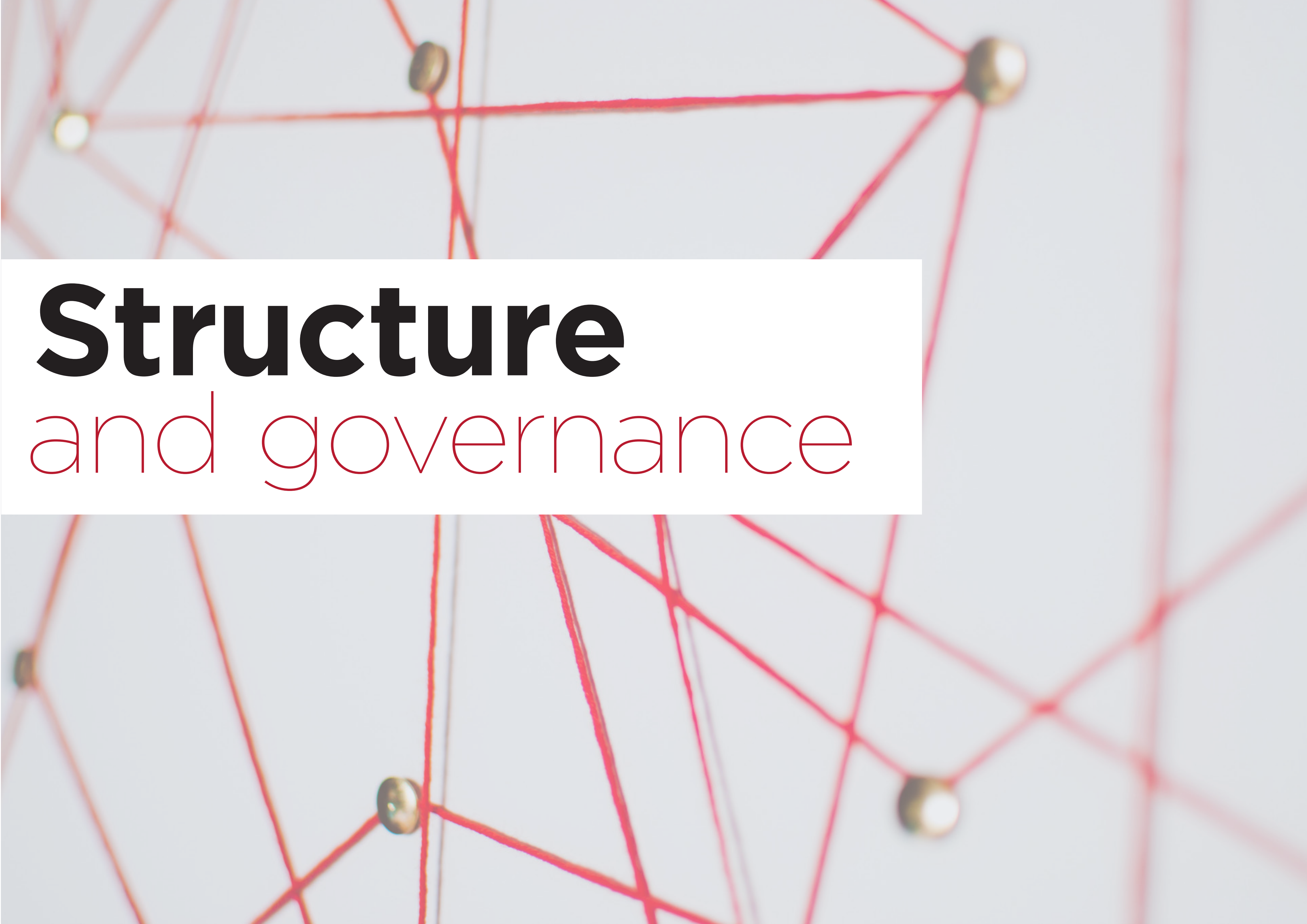
Ensure constructive ongoing partnership and engagement between the EHC SC, MAG and MASAG

As complementary bodies in the governance and strategic work of the organisation, the EHC takes care to ensure a constructive and ongoing dialogue between the elected members of the SC – composed of patients or their family members – and the appointed members of the MAG and MASAG – both composed of health care professionals and other external experts. All parties are guided by a Terms of Reference and hold frequent joint meetings to ensure maximum dialogue, collaboration and alignment on key issues.

Ensure adequate staff and financial resources to implement the strategic plan

In 2017, the EHC continued to expand its focus in order both to adapt to the gradually changing health care paradigm and to continue to address the ongoing challenges of the rare bleeding disorders community. In order to ensure its growth is met with the necessary support, the organisation hired a new staff position for communications work, previously carried out simultaneously by the public policy officer. Additionally, the EHC hired a consultant to carry out, coordinate and help implement its PARTNERS programme.

The EHC closed 2017 with more than €300,000 in positive income over expenditures, allowing it to strengthen its operational reserves, solidify its already established activities and create new ones where needed.



Structure and governance

Steering Committee (SC)

The EHC is governed by a General Assembly composed of representatives from all National Member Organisations (NMOs). Between General Assemblies, the EHC's activities are overseen by a Steering Committee (SC) elected by the General Assembly and composed of a President, Vice-President Finance and SC members. All SC member are elected to a four-year term.

The SC is composed of:

- Brian O'Mahony, EHC President, Irish NMO
- Jordan Nedevski, EHC Vice-President Finance, Bulgarian NMO
- Radoslaw Kaczmarek, EHC SC Member, Polish NMO
- Olivia Romero-Lux, EHC SC Member, French NMO
- Naja Skouw-Rasmussen, EHC SC Member, Danish NMO
- Miguel Crato, EHC SC Member, Portuguese NMO
- Giuseppe Mazza, co-opted SC Member, Italian NMO (not pictured)
- Alain Weill, Ex-Officio, WFH President
- Amanda Bok, Ex-Officio, EHC CEO

Medical Advisory Group (MAG)

Since its establishment in 2009, the MAG has provided high-level medical and scientific support to the SC and the EHC staff. It is composed of European medical and scientific experts in haemophilia and other congenital bleeding disorders. The MAG is composed of:

- Prof Paul Giangrande (Chairperson), Oxford University
- Prof Angelika Batorova, Medical Director of the National Haemophilia Centre and the Haemostasis and Thrombosis Unit, University Hospital, Bratislava
- Prof Michael Makris, Honorary Consultant Haematologist, Sheffield Teaching Hospitals, NHS Trust
- Prof Flora Peyvandi, Director of the Department of Internal Medicine, RCCS Maggiore Hospital, University of Milan

Creation of the Medical and Scientific Advisory Group

Close collaboration with a comprehensive team of medical experts is essential to the work of the EHC. In addition to the already established Medical Advisory Group (MAG), in 2017, the EHC formed the Medical and Scientific Advisory Group (MASAG) with the objective to:

- Help ensure a multidisciplinary approach to the work of the EHC
- Provide advice and guidance on specific areas of EHC engagement
- Provide strategic input into specific event programmes and activities as necessary
- Provide hands-on, in person support in key events as needed.

The group is composed of 16 health care professionals from different areas of expertise.

EHC Committees

YOUTH COMMITTEE

The EHC Youth Committee develops the programme of the EHC Youth Leadership Workshop and provides advice to the SC on youth-related matters. It is supported by Amanda Bok, EHC CEO and Kristine Jansone, EHC Inhibitor Programme Officer.

The EHC Youth Committee is composed of:

Chair – Michael van der Linde	Dutch NMO
Amanda Bok	EHC CEO
Aleksandra Ilijn	Serbian NMO
Kristine Jansone	EHC Inhibitor Programme Officer
Olivia Romero-Lux	EHC SC Member / French NMO
Thomas Sannié	French NMO
Ivan Sebest	Slovakian NMO
Traci Marshall-Dowling	Irish NMO

Women’s Working Group

During the 2016 EHC Conference, NMOs with an interest in women's activities held a meeting to exchange information on national activities organised for women with bleeding disorders, carriers, and carers or partners of a person with a bleeding disorder.

The meeting led to the creation of an EHC Women and Bleeding Disorders Working Group in 2017, which aimed to create awareness, recognition, support and education about and for women in the bleeding disorders community.

The group established an activity plan for the coming years, which was accepted by the EHC Steering Committee in February, and includes:

- Communication and raising awareness about carriers with symptoms of bleeding disorders and their quality of life
- Education of doctors
- Recognition
- Supporting newly diagnosed families
- Education within and outside of NMOs
- Addressing medical questions, gynaecological issues, treatment options, puberty (menorrhagia), prenatal diagnosis, pregnancy and childbirth
- Improving psychological and social well-being
- Emotional support

Throughout the year, the group focused on seeking information from all EHC NMOs in order to build a European network and be able to work on ways to address the challenges of women with rare bleeding disorders.

INHIBITOR WORKING GROUP (IWG)

The IWG supports the planning, development and creation of the European Inhibitor Network.

It is composed of:

Amanda Bok	EHC CEO
Christina Burgess	UK NMO
Miguel Crato	Portuguese NMO
Paul Giangrande	EHC MAG Chair / Oxford University
Carl Fredrik Gustafsson	Swedish NMO
Radoslaw Kaczmarek	EHC SC / Polish NMO
Kristine Jansone	EHC Inhibitor Programme Officer
Mirko Jokic	Serbian NMO
Hannele Kareranta	Finnish NMO
Teresa Pereira	Portuguese NMO

Not Pictured: Carl Fredrik Gustafsson, Hannele Kareranta and Terese Pereira

NEWSLETTER EDITORIAL COMMITTEE

The Newsletter Editorial Committee supports the EHC with the conception and planning of content and production of the EHC newsletter.

The EHC wishes to thank all NMOs and external contributors who submitted material for publication in the newsletter.

Amanda Bok	EHC CEO
Radoslaw Kaczmarek	EHC SC Member/ Polish NMO
Olivia Romero-Lux	EHC SC member / French NMO
Laura Savini	EHC Public Policy Officer
Raia Mihaylova	EHC Communications Officer

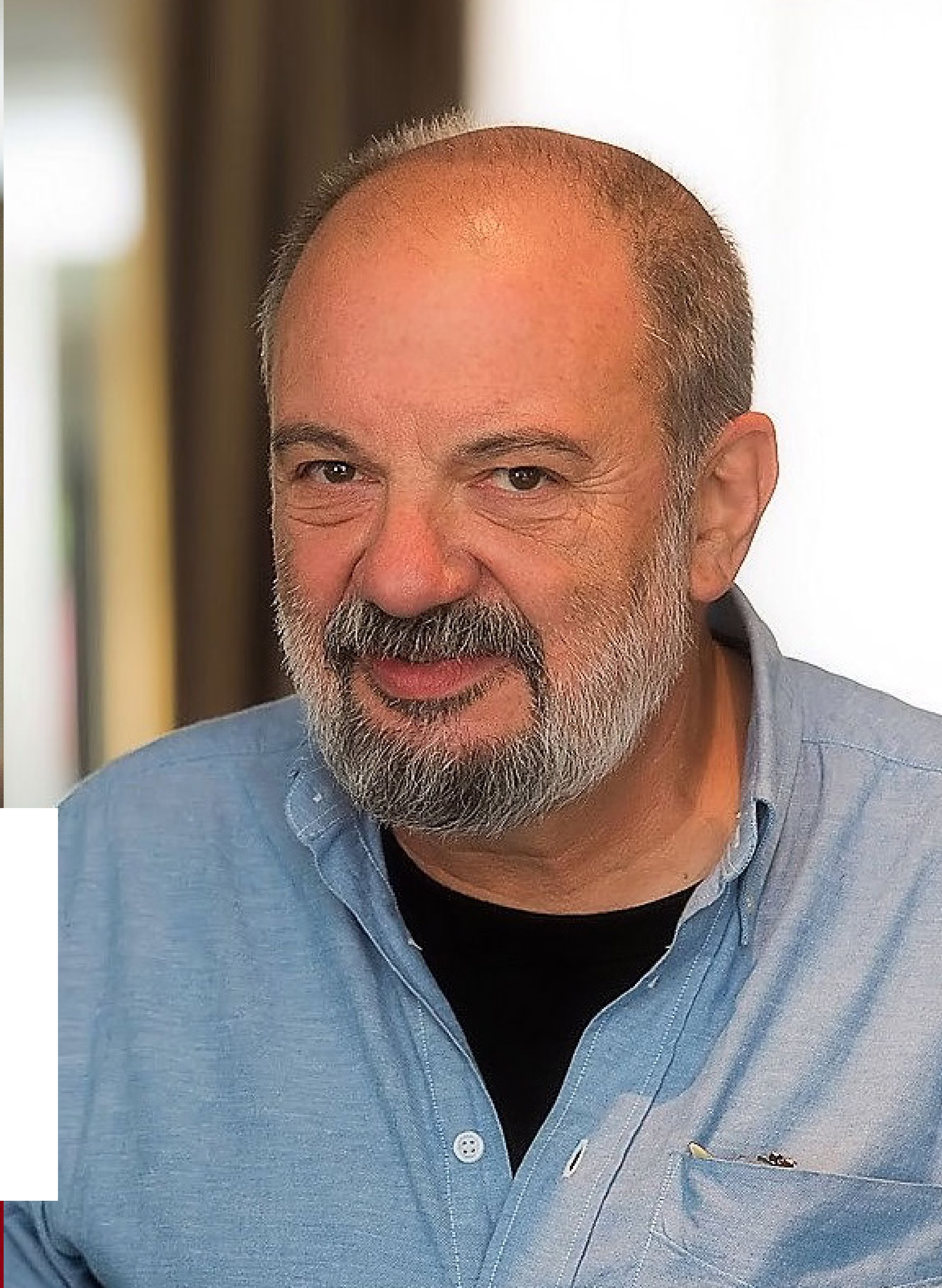
STAFF

To support its growing work, in 2017 the EHC expanded its team and welcomed Ms Raia Mihaylova as a Communications Officer and Mr Declan Noone as EHC PARTNERS programme consultant.

The EHC office is based in Brussels with staff members:

Amanda Bok	EHC CEO
Jo Eerens	EHC Membership Officer
Kristine Jansone	EHC Inhibitor Programme Officer
Raia Mihaylova	EHC Communications Officer
Declan Noone	EHC PARTNERS programme consultant
Saskia Pfeyffer	EHC Office Assistant
Laura Savini	EHC Public Policy Officer

Financial Report



Message from the Vice-President Finance and CEO

Drawing this Annual Report to a close, we wish to echo the sentiments that opened it, namely that each year we see a more dynamic and vibrant community being active at the EHC.

Nowhere is this more evident than in the numbers (!) - particularly those of volunteer in-kind contributions. These have grown steadily from year to year since we started keeping volunteer records in 2014 and reported the equivalent of €244,380 donated by volunteers to the EHC through their non-remunerated work for our organisation. Reporting €302,000 of volunteer in-kind contributions in 2017, we take this moment to thank all of our volunteers for the 24% increase in their time and dedication over the past three years. Their input clearly reflects in the ongoing - and growing - quality of the EHC's work!

And the increase in interest and engagement does not stop with volunteers but extends into all stakeholders. The strongest quantitative measure of this can be found in the EHC Conference accounts, which in 2017 closed with a historic profit of €130,848 - 35% higher than the year before (which itself saw a 10% growth compared to its previous iteration).

Yet we haven't been sitting on these happy and healthy financial laurels - we have been investing them! In 2017 we hired two new and additional human resources, a PARTNERS programme consultant and a Communications Officer (thereby splitting the previously joint Communications and Public Policy position into two separate and full-time jobs). We also got out our wheels, carton boxes and IKEA tools, and moved ourselves into a larger office (in the same building) to accommodate our growing office team.

Still, we remained prudent, ending 2017 with positive financial results of €309,661 in surplus to put towards new initiatives and towards continuing to maintain a healthy financial reserve, which itself must inevitably continue to grow in order to match an upwardly shifting annual budget.

As you can see, we are pleased to report another productive and positive year, and in the next pages invite you to review our final 2017 accounts and external audit in more detail.

But before you do, we leave you with a note of gratitude - for the wonderful work of our volunteers and professional staff, for the ongoing and growing trust and support of our funding partners, and most importantly, for the moving and rewarding fruits of our collective labour, which we see growing more engaged and more empowered every day in our vibrant and dynamic European community.

Jordan Nedevski
Vice-President Finance

Amanda Bok
CEO

Report of the auditor

Rosier & Co
Réviseurs d'entreprises

INDEPENDENT AUDITOR’S REPORT TO THE STEERING COMMITTEE
OF THE AISBL

Consortium Européen de l’Hémophilie
(European Haemophilia Consortium)
0887.106.966

FOR THE YEAR ENDED 31 DECEMBER 2017

As required, we report to you in the context of our appointment as the association’s independent auditor. This report includes our opinion on the “internal statements”, further the annual accounts.

Report on the annual accounts – Unqualified opinion

We have audited the annual accounts of the AISBL *Consortium Européen de l’Hémophilie* for the year ended 31 December 2017, prepared in accordance with the financial-reporting framework applicable in Belgium, which show a balance sheet total of € 3.100.697,90 and a positive result for the year of € 309.661,29.

Responsibility of the board of Directors for the preparation of the annual accounts

The board of Directors is responsible for the preparation of annual accounts that give a true and fair view in accordance with the financial-reporting framework applicable in Belgium, and for such internal control as the board of Directors determines is necessary to enable the preparation of annual accounts that are free from material misstatement, whether due to fraud or error.

Responsibility of the statutory auditor

Our responsibility is to express an opinion on these annual accounts based on our audit. We conducted our audit in accordance with International Standards on Auditing (ISAs). Those standards require that we comply with the ethical requirements and plan and perform the audit to obtain reasonable assurance about whether the annual accounts are free from material misstatement.

An audit involves performing procedures to obtain audit evidence about the amounts and disclosures in the annual accounts. The procedures selected depend on the independent auditor's judgment, including the assessment of the risks of material misstatement of the annual accounts, whether due to fraud or error. In making those risk assessments, the independent auditor considers the company’s internal control relevant to the preparation of annual accounts that give a true and fair view, in order to design audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the entity’s internal control. An audit also includes evaluating the appropriateness of accounting policies used and the reasonableness of accounting estimates made by the board of Directors, as well as evaluating the overall presentation of the annual accounts.

We have obtained from the board of Directors and company officials the explanations and information necessary for performing our audit.

We believe that the audit evidence we have obtained is sufficient and appropriate to provide a basis for our opinion.

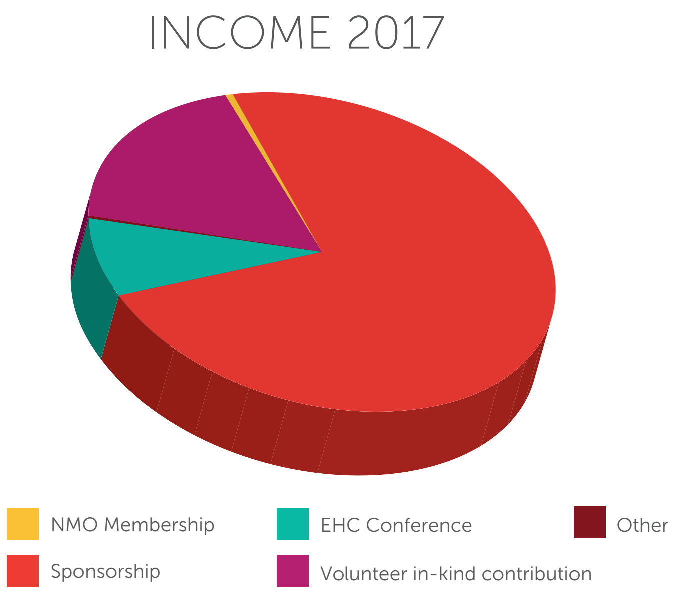
Unqualified opinion

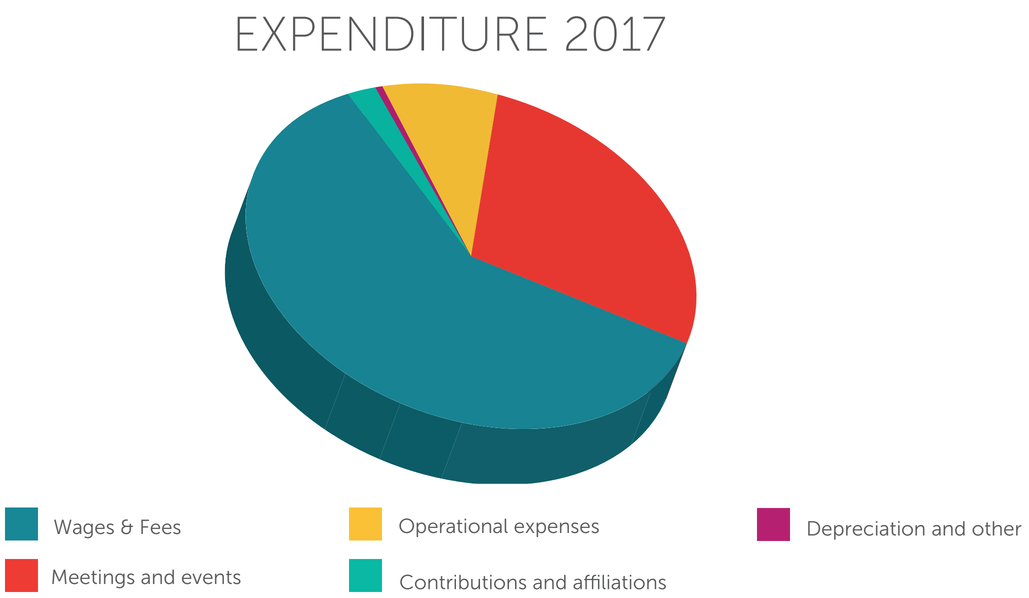
In our opinion, the annual accounts of the AISBL’s give a true and fair view of the association’s equity and financial position as at 31 December 2017, and of the results of its operations for the year then ended, in accordance with the financial-reporting framework applicable in Belgium.

Limal, June 26th 2018

Rosier & Co
Independent auditor's
Stéphane Rosier
represented by
Stéphane Rosier
Certified Public Auditor

Income and expenditure

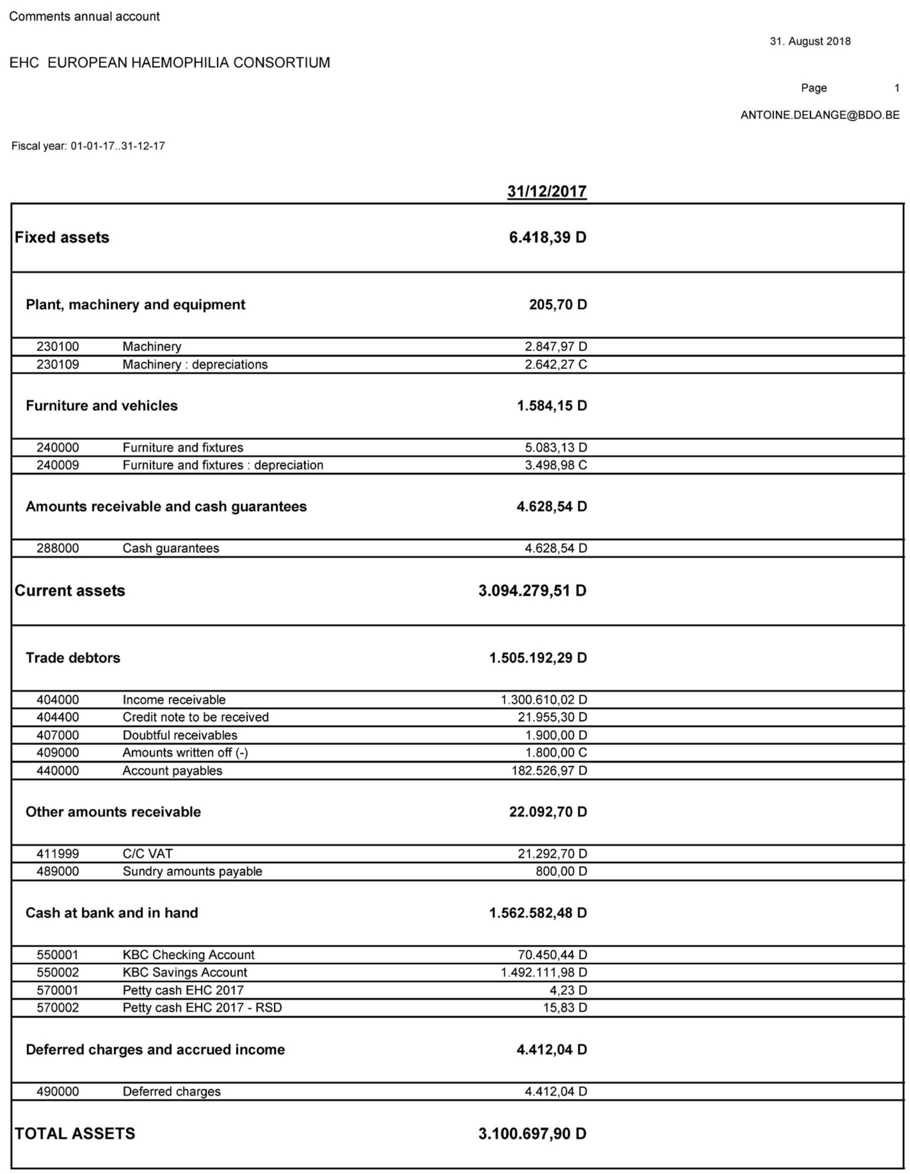




INCOME	FINALS (€)
NMO Membership	10,850
Sponsorship	1,215,478
EHC Conference	130,848
Other	5,157
SUB-TOTAL INCOME	1,362,333
Volunteer in-kind contribution	302,000
TOTAL INCOME	1,664,333

EXPENDITURE	FINALS (€)
Operational expenses	89,331
Wages and fees	641,681
Meetings and events	293,048
Contributions and affiliations	23,821
Depreciation	4,791
TOTAL EXPENDITURE	1,052,672

Balance Sheet



Comments annual account

31. August 2018

EHC EUROPEAN HAEMOPHILIA CONSORTIUM

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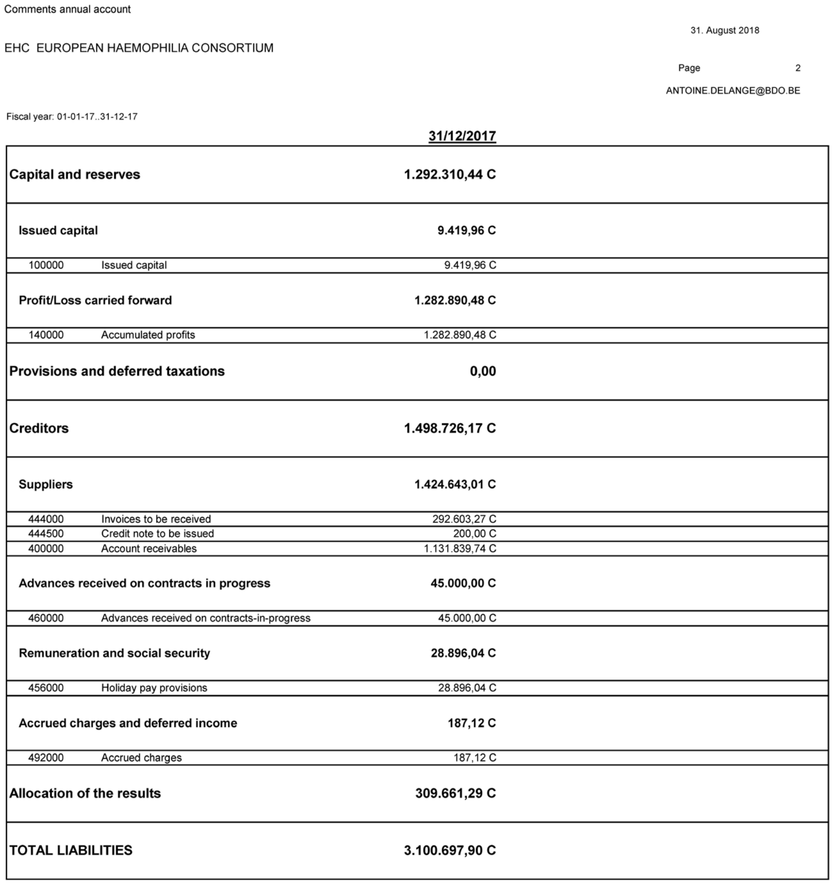
Fiscal year: 01-01-17..31-12-17

		31/12/2017
Fixed assets		**6.418,39 D**
Plant, machinery and equipment		**205,70 D**
230100	Machinery	2.847,97 D
230109	Machinery : depreciations	2.642,27 C
Furniture and vehicles		**1.584,15 D**
240000	Furniture and fixtures	5.083,13 D
240009	Furniture and fixtures : depreciation	3.498,98 C
Amounts receivable and cash guarantees		**4.628,54 D**
288000	Cash guarantees	4.628,54 D
Current assets		**3.094.279,51 D**
Trade debtors		**1.505.192,29 D**
404000	Income receivable	1.300.610,02 D
404400	Credit note to be received	21.955,30 D
407000	Doubtful receivables	1.900,00 D
409000	Amounts written off (-)	1.800,00 C
440000	Account payables	182.526,97 D
Other amounts receivable		**22.092,70 D**
411999	C/C VAT	21.292,70 D
489000	Sundry amounts payable	800,00 D
Cash at bank and in hand		**1.562.582,48 D**
550001	KBC Checking Account	70.450,44 D
550002	KBC Savings Account	1.492.111,98 D
570001	Petty cash EHC 2017	4,23 D
570002	Petty cash EHC 2017 - RSD	15,83 D
Deferred charges and accrued income		**4.412,04 D**
490000	Deferred charges	4.412,04 D
TOTAL ASSETS		**3.100.697,90 D**



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EHC EUROPEAN HAEMOPHILIA CONSORTIUM

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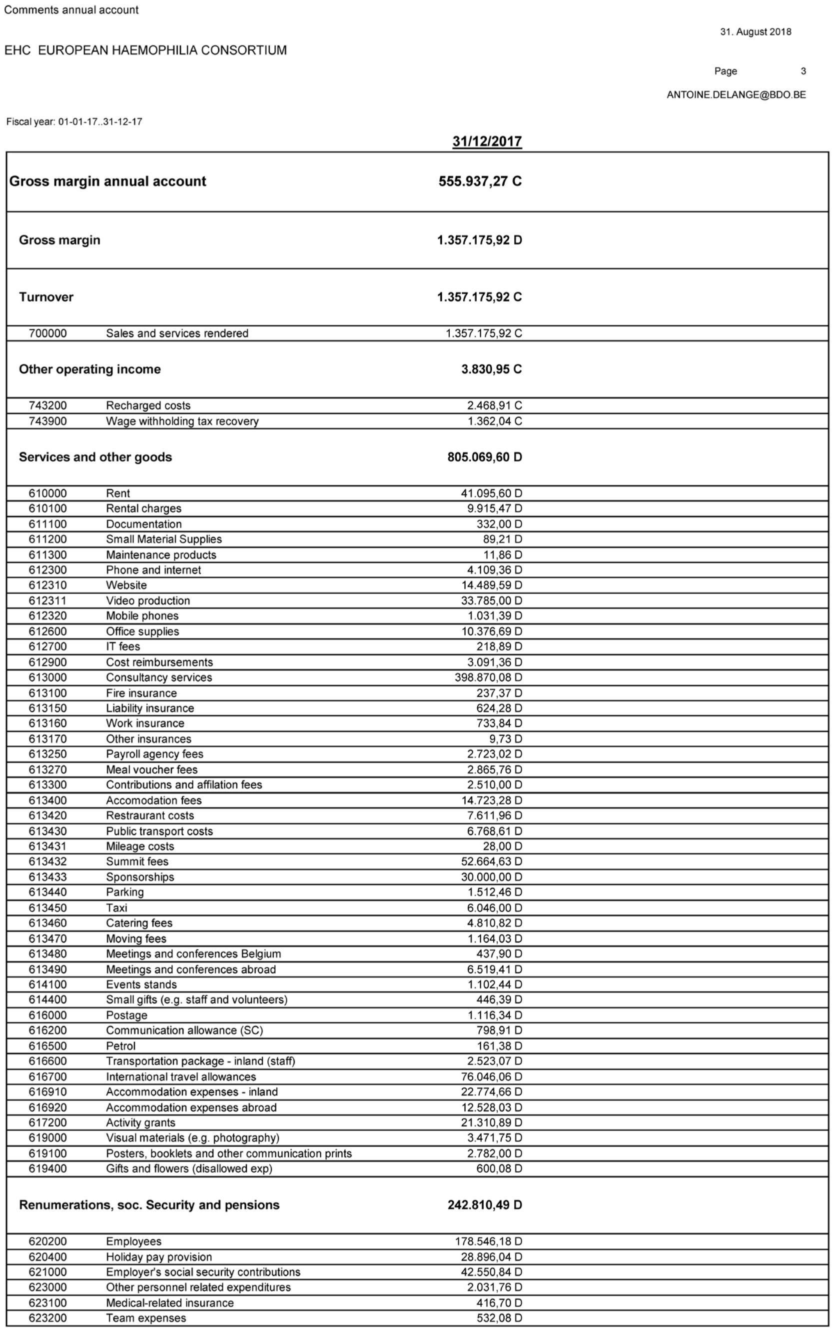
Fiscal year: 01-01-17..31-12-17

		31/12/2017
Capital and reserves		**1.292.310,44 C**
Issued capital		**9.419,96 C**
100000	Issued capital	9.419,96 C
Profit/Loss carried forward		**1.282.890,48 C**
140000	Accumulated profits	1.282.890,48 C
Provisions and deferred taxations		**0,00**
Creditors		**1.498.726,17 C**
Suppliers		**1.424.643,01 C**
444000	Invoices to be received	292.603,27 C
444500	Credit note to be issued	200,00 C
400000	Account receivables	1.131.839,74 C
Advances received on contracts in progress		**45.000,00 C**
460000	Advances received on contracts-in-progress	45.000,00 C
Remuneration and social security		**28.896,04 C**
456000	Holiday pay provisions	28.896,04 C
Accrued charges and deferred income		**187,12 C**
492000	Accrued charges	187,12 C
Allocation of the results		**309.661,29 C**
TOTAL LIABILITIES		**3.100.697,90 C**



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EHC EUROPEAN HAEMOPHILIA CONSORTIUM

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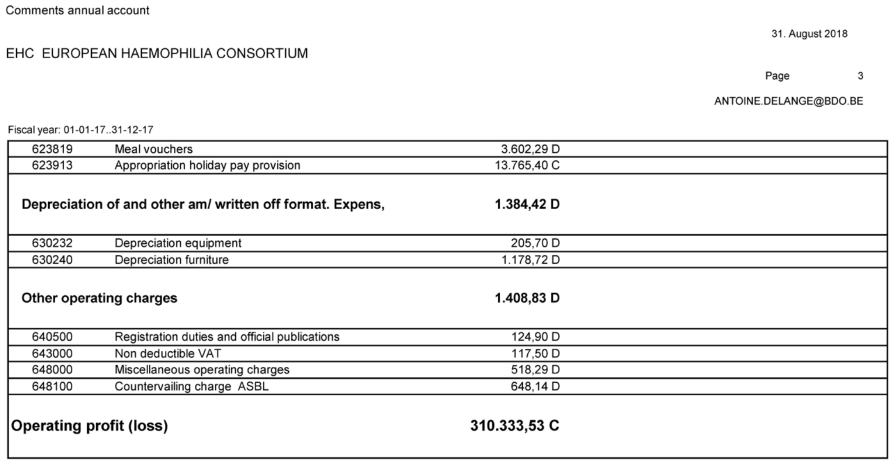
Fiscal year: 01-01-17..31-12-17

		31/12/2017
Gross margin annual account		**555.937,27 C**
Gross margin		**1.357.175,92 D**
Turnover		**1.357.175,92 C**
700000	Sales and services rendered	1.357.175,92 C
Other operating income		**3.830,95 C**
743200	Recharged costs	2.468,91 C
743900	Wage withholding tax recovery	1.362,04 C
Services and other goods		**805.069,60 D**
610000	Rent	41.095,60 D
610100	Rental charges	9.915,47 D
611100	Documentation	332,00 D
611200	Small Material Supplies	89,21 D
611300	Maintenance products	11,86 D
612300	Phone and internet	4.109,36 D
612310	Website	14.489,59 D
612311	Video production	33.785,00 D
612320	Mobile phones	1.031,39 D
612600	Office supplies	10.376,69 D
612700	IT fees	218,89 D
612900	Cost reimbursements	3.091,36 D
613000	Consultancy services	398.870,08 D
613100	Fire insurance	237,37 D
613150	Liability insurance	624,28 D
613160	Work insurance	733,84 D
613170	Other insurances	9,73 D
613250	Payroll agency fees	2.723,02 D
613270	Meal voucher fees	2.865,76 D
613300	Contributions and affilation fees	2.510,00 D
613400	Accomodation fees	14.723,28 D
613420	Restraurant costs	7.611,96 D
613430	Public transport costs	6.768,61 D
613431	Mileage costs	28,00 D
613432	Summit fees	52.664,63 D
613433	Sponsorships	30.000,00 D
613440	Parking	1.512,46 D
613450	Taxi	6.046,00 D
613460	Catering fees	4.810,82 D
613470	Moving fees	1.164,03 D
613480	Meetings and conferences Belgium	437,90 D
613490	Meetings and conferences abroad	6.519,41 D
614100	Events stands	1.102,44 D
614400	Small gifts (e.g. staff and volunteers)	446,39 D
616000	Postage	1.116,34 D
616200	Communication allowance (SC)	798,91 D
616500	Petrol	161,38 D
616600	Transportation package - inland (staff)	2.523,07 D
616700	International travel allowances	76.046,06 D
616910	Accommodation expenses - inland	22.774,66 D
616920	Accommodation expenses abroad	12.528,03 D
617200	Activity grants	21.310,89 D
619000	Visual materials (e.g. photography)	3.471,75 D
619100	Posters, booklets and other communication prints	2.782,00 D
619400	Gifts and flowers (disallowed exp)	600,08 D
Renumerations, soc. Security and pensions		**242.810,49 D**
620200	Employees	178.546,18 D
620400	Holiday pay provision	28.896,04 D
621000	Employer's social security contributions	42.550,84 D
623000	Other personnel related expenditures	2.031,76 D
623100	Medical-related insurance	416,70 D
623200	Team expenses	532,08 D



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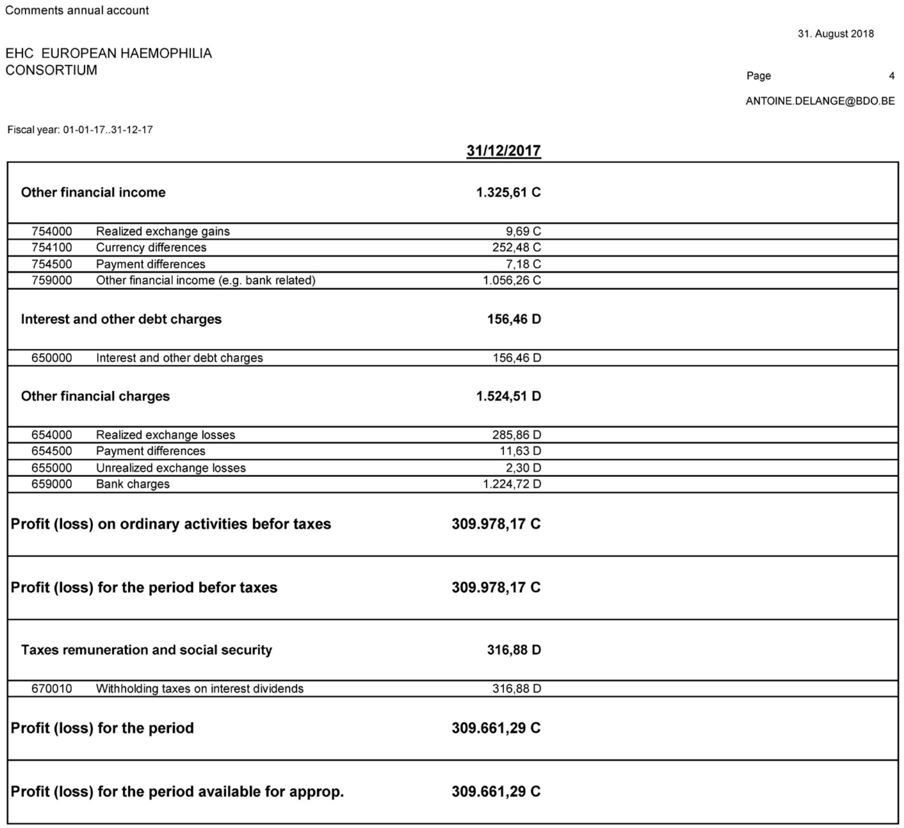
Fiscal year: 01-01-17..31-12-17

623819	Meal vouchers	3.602,29 D
623913	Appropriation holiday pay provision	13.765,40 C
Depreciation of and other am/ written off format. Expens,		**1.384,42 D**
630232	Depreciation equipment	205,70 D
630240	Depreciation furniture	1.178,72 D
Other operating charges		**1.408,83 D**
640500	Registration duties and official publications	124,90 D
643000	Non deductible VAT	117,50 D
648000	Miscellaneous operating charges	518,29 D
648100	Countervailing charge ASBL	648,14 D
Operating profit (loss)		**310.333,53 C**



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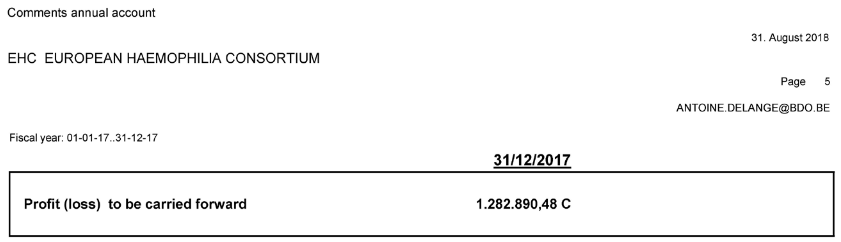
Fiscal year: 01-01-17..31-12-17

		31/12/2017
Other financial income		**1.325,61 C**
754000	Realized exchange gains	9,69 C
754100	Currency differences	252,48 C
754500	Payment differences	7,18 C
759000	Other financial income (e.g. bank related)	1.056,26 C
Interest and other debt charges		**156,46 D**
650000	Interest and other debt charges	156,46 D
Other financial charges		**1.524,51 D**
654000	Realized exchange losses	285,86 D
654500	Payment differences	11,63 D
655000	Unrealized exchange losses	2,30 D
659000	Bank charges	1.224,72 D
Profit (loss) on ordinary activities befor taxes		**309.978,17 C**
Profit (loss) for the period befor taxes		**309.978,17 C**
Taxes remuneration and social security		**316,88 D**
670010	Withholding taxes on interest dividends	316,88 D
Profit (loss) for the period		**309.661,29 C**
Profit (loss) for the period available for approp.		**309.661,29 C**



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Fiscal year: 01-01-17..31-12-17

	31/12/2017
Profit (loss) to be carried forward	**1.282.890,48 C**

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Putting hemophilia patients first

yesterday, today, tomorrow

At Bayer, we're all about helping people with hemophilia to break boundaries, thrive and achieve their full potential.

We've been doing this for nearly 30 years and are proud to work with patients, caregivers and treaters to help create a strong community dedicated to reaching these goals.

But we never stop.

Our portfolio of innovative recombinant FVIII therapies is currently setting new standards of care. And, our evolving patient support programs and tireless research into gene therapy and bleeding disorders, is helping redefine what it means to live with hemophilia.

***Breaking boundaries** in hemophilia*


changing haemophilia™
~~limited~~ more
treatment choice
CHRIS BOMBARDIER
Chris lives in the US
and has haemophilia B


changing haemophilia™
~~limited~~ improved
access to care
BRIAN SOUCY
Brian lives in the US
and has haemophilia


changing haemophilia™
~~limited~~ increased
mobility
LIAM CLEMENS
Liam lives in the US
and has haemophilia A

Building on our Heritage to *Pioneer a New Era* of Care

Pfizer Haemophilia

Global commitment to patients through community initiatives

Pfizer believes in partnering with the haemophilia community at all levels to support initiatives that aim to improve access, standards of care and education for people with haemophilia and their families

Funding from Pfizer has contributed to the following EHC-organised activities:*

- **Economic workshops**
- **Round tables**
- **Leadership conferences**

PHYSIOTHERAPY SCHOOL FOR HAEMOPHILIA

Physiotherapy School for Haemophilia aims to advance the science and understanding of the role of physiotherapy in improving outcomes for people with haemophilia†

The **Pfizer Ultrasound Programme** aims to support initiatives to provide education, awareness, research support and hands-on training in the use of point-of-care ultrasound†

Pfizer Ultrasound Programme
Innovation and Imaging in Haemophilia

EHC 2018

This year, the **Pfizer-sponsored Satellite Symposium** at the EHC 2018 Conference has been developed in collaboration with EHC. The symposium has been designed to help raise awareness around the value of physiotherapy in haemophilia.

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Date of preparation: August 2018

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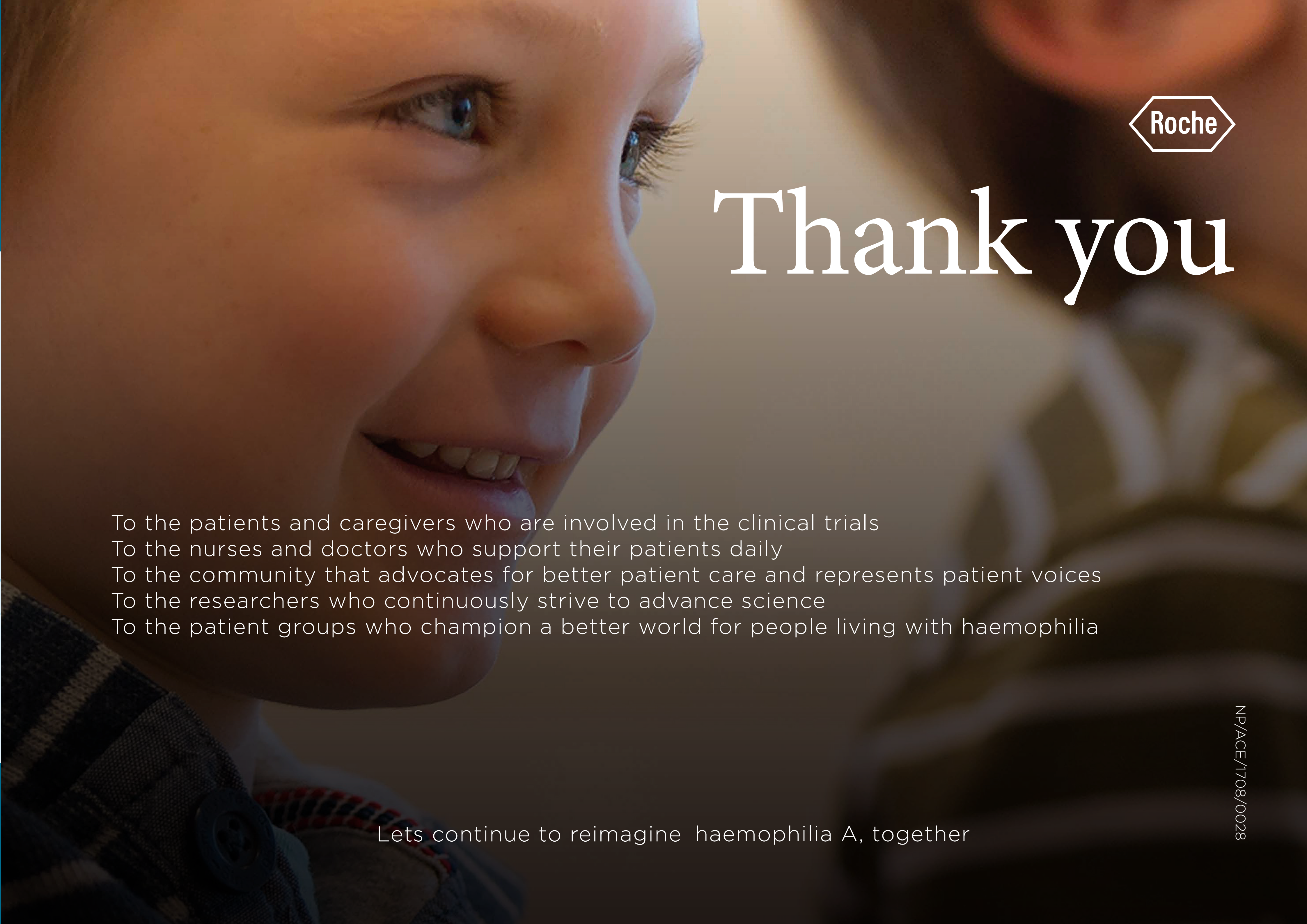
*These activities are initiated and organised by EHC. The content and activities described are independently developed by the society. Pfizer is not the sole commercial sponsor for EHC.

†These programmes have been initiated and funded by Pfizer.

To find out more about our support service and tools, contact your local Pfizer team.



Thank you

To the patients and caregivers who are involved in the clinical trials
To the nurses and doctors who support their patients daily
To the community that advocates for better patient care and represents patient voices
To the researchers who continuously strive to advance science
To the patient groups who champion a better world for people living with haemophilia

Lets continue to reimagine haemophilia A, together

NP/ACE/1708/0028

Working together to ensure sustainable access

At Sobi, we are proud to be working with governments and organisations such as the European Haemophilia Consortium to provide sustainable access to treatments for haemophilia. To achieve this, we collaborate with member organisations, governments and healthcare providers to find the best long term solution for people with haemophilia based on conditions in the countries where they live.

The EHC's PARTNERS programme (Procurement of Affordable Replacement Therapies – Network of European Relevant Stakeholders) is a perfect example of a joint initiative designed to provide treatment for people with haemophilia in a specific region. The PARTNERS programme is bridging the gap between the recognized minimum haemophilia treatment recommendations and the volume of therapy available in several European countries which have developed healthcare systems but are limited by highly restricted budgets.

One of the main goals of PARTNERS is to increase access to replacement therapies in European countries that provide little or no such treatment to their haemophilia patients. More than 5,000 people with haemophilia are expected to benefit from the programme.

Our work with PARTNERS is part of Sobi's broader commitment to improving access to treatment for people with haemophilia, which also includes extensive support of the World Federation of Hemophilia's Humanitarian Aid Program. We are committed to working together with the EHC and other partners to ensure that as many people as possible benefit from increased, sustainable access to treatments that can transform their lives.

Read more about our humanitarian programmes at www.sobi.com and about the PARTNERS programme at www.ehc.eu/partners/

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THE SOUND OF WHAT'S NEXT IN HAEMOPHILIA
ON
OFF

EHC

european haemophilia consortium

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