# 2016
# ANNUAL REPORT
## EUROPEAN HAEMOPHILIA CONSORTIUM

EHC
european haemophilia consortium

## ABREVIATIONS EHC

**CEO:** Chief Executive Officer
**CoE:** Council of Europe
**DAA:** direct-acting antivirals
**DEC:** Data and Economics Committee
**EAHAD:** European Association for Haemophilia and Allied Disorders
**EASL:** European Association for the Study of the Liver
**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
**EPF:** European Patients' Forum
**HCV:** hepatitis C virus
**IU:** international unit
**IWG:** Inhibitor Working Group
**MAG:** Medical Advisory Group
**MEP:** member of the European Parliament
**MoU:** Memorandum of Understanding
**NMO:** National Member Organisation
**PLUS:** Platform for plasma Users
**PUP:** previously untreated patient
**PWH:** people with haemophilia
**PWI:** people with inhibitors
**SC:** Steering Committee
**SIPPET:** Survey of Inhibitors in Plasma-Product Exposed Toddlers
**UNDP:** United Nations Development Programme
**WFH:** World Federation of Hemophilia

# TABLE OF CONTENTS

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## 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 and other rare bleeding disorders across Europe.

## Mission Statement

The EHC was established in 1989 with the mission to:

- Ensure adequate supply of – 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


STAGEWORK
New York
DEUS
SWEET
SKTBS

# Members

The EHC has 45 National Member Organisations (NMOs) from 27 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

In some ways, 2016 distinguishes itself from its preceding years.

It is the first year since we took up our roles as President and CEO that the EHC did not implement a major new program or activity in our organisational arsenal but rather took the opportunity to pause, tune in internally and focus on trees over forest by spotlighting the needs of PWH who are ageing; PWH who are infected with hepatitis C (HCV); PWH with inhibitors (PWI) and their caregivers – all of whom struggle, often in isolation and frequently invisibly; and PWH living in Eastern and Central European countries with endemic lack of adequate access to treatment.

In 2016, we pulled back the curtains on individual European lives, replaced data with faces, launched the 'Haemophilia Stories' documentary, dedicated our World Haemophilia Day Round Table events to inhibitors, ageing, HCV and personalised outcome measures, and received resounding endorsement from our members to start 2017 with an access programme called PARTNERS (which stands for Procurement of Affordable Replacement Therapies – Network of European Relevant Stakeholders). We launched a survey on PWH and HCV and held our first Inhibitor Summit (part of the European Inhibitor Network programme launched in 2015) for PWI, their families and caregivers. 2016 was about people; it was about the diversity of our community and about how we intend to meet the divergent remaining needs in Europe.

By broadening and deepening our existing programmes and activities, we almost doubled our expenditures in 2016 by focusing more on our membership and on ways to support them, adding – for example – the opportunity for our National Member Organisations (NMOs) to apply for Activity Grants to cover gaps in their national sponsorship opportunities. We also introduced a new focus to our economics workshop series and started training patient representatives on how to become formally and officially involved in their national tender and procurement processes.

By focusing on people, we grew our partnership base significantly in 2016, increasing our sponsorship support by 60% and growing our collaboration with internal and external volunteers. We signed a Memorandum of Understanding (MoU) with the United Nations Development Programme (UNDP) in Ukraine, connected both with the European Liver Patients' Association (ELPA) and the European Association for the Study of the Liver (EASL), and added to the EHC staff base by welcoming Saskia Pfeyffer to the Brussels-based team as our new Office Assistant.

We are proud of our collective achievements in 2016, as indeed of all the years before it; we are quickly becoming the organisation that we envisaged and invite you to read more about our work in the next pages, and to join us in our continued efforts.

Brian o Mahony

Brian O'Mahony
*President*

Amanda Bok
*CEO*

# EHC 2016 activities at a glance

## HIGHLIGHTS FROM THE YEAR:

- Organised World Haemophilia Day events to raise awareness on issues of access to treatment and care in Europe
- Elections of lay and new Steering Committee (SC) members
- EHC Conference in Stavanger, Norway
- Laid out the framework for PARTNERS programme to increase access to treatment in 12 countries
- Held the first EHC European Inhibitor Summit
- Signed MoU with the UNDP Ukraine
- Worked with key European health care providers to lay the framework for developing 'European Principles on Inhibitor Management'
- Added a new staff member to the EHC team

## MEDICAL AND SCIENTIFIC UPDATE ON:

- Inhibitors in haemophilia A
- HCV treatment
- Care for the ageing haemophilia population
- Outcome measures in haemophilia
- New technologies in haemophilia care

## TRAINING AND BEST PRACTICES IN:

- Tenders and Procurement – patient and clinician involvement
- NMO management, strategic planning and funding
- Engagement of patients with inhibitors
- Communication skills and engagement of young volunteers

## DATA COLLECTION ON:

- HCV and haemophilia – number of diagnosed people, their clinical outcomes and access to HCV testing and treatment

**FEBRUARY**
Round Table on Inhibitors in Haemophilia A

**APRIL**
Youth Leadership Workshop;
World Hemophilia Day events;
Round Table on Ageing and Haemophilia

**JUNE**
Leadership Conference;
Round Table on HCV and Haemophilia

**SEPTEMBER**
Tenders and Procurement Workshop

**OCTOBER**
General Assembly
EHC Conference;

**NOVEMBER**
Workshop on New Technologies in Haemophilia Care;
Round Table on Personalised Outcome Measures in Haemophilia

**DECEMBER**
First European Inhibitor Summit


axalta
Now part of Shire

# Activity Report

**In 2016, the EHC continued to carry out activities in line with its 2014-2017 Strategic Plan objectives, which seek 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 environment in which the EHC operates.

# SUPPORT AND EMPOWER NMOs

The EHC was established by European haemophilia patient organisations (which now form its NMOs) to serve them both on a national and European level. To do so, the EHC has made it a core strategy to support and strengthen NMO work by providing updated information on health policies, legislative and scientific developments, targeted trainings, advocacy materials and best practice exchanges between patient communities.

The EHC also collects annual data to assess different aspects of haemophilia care in Europe and target the areas that need improvement. The results are used to develop advocacy materials that empower members to act on a local level.

# High-quality, timely communication

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

## WEBSITE

- Contains information on the EHC, its events and initiatives
- Regularly updated with key developments in the field of rare bleeding disorders
- Over 25,000 unique visits in 2016

## QUARTERLY 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 and event pictures
- Posts that highlight NMO activities
- Links to useful articles that deal with the daily challenges of rare bleeding disorders

## NEWSLETTER

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

## EVENTS REPORTS

- Produced following Round Tables to report in detail on the presentations and discussions held during the event

## YOUTUBE CHANNEL

- Used to disseminate EHC advocacy videos and the EHC Annual Conference scientific sessions webcasts

# Education and training

The EHC organises various annual events to provide its NMOs with the latest information on scientific and medical developments in the field of haemophilia and other rare bleeding disorders. The organisation also provides targeted trainings to its members and encourages participation in events by providing travel and accommodation grants, including to its Brussels-based Round Tables, the EHC Conference and World Haemophilia Day event. In the section below is a brief description of each event.

## ROUND TABLE ON INHIBITORS IN HAEMOPHILIA A

*February 2016 – Brussels, Belgium*

For approximately 30% of people with severe haemophilia A (factor VIII deficiency), traditional replacement therapy becomes ineffective and a major change in clinical management is required. This complication is known as inhibitor development, where the immune system stops recognising, and therefore destroys, the infused factor concentrate. Bleeds become more difficult to treat and patients are at an increased risk of disability and death. To manage inhibitors, patients have to resort to more expensive and challenging approaches, such as bypassing agents and immune tolerance induction.

Inhibitor development is one of the leading challenges in haemophilia care today. Though scientists have not yet identified the exact reason for its formation, there have been significant research advances in recent years. In light of these updates and to facilitate important discussions on the topic, the EHC held its first Round Table of Stakeholders of the year on Inhibitors in Haemophilia A.

The event gathered over 75 participants including patient representatives, leading health care professionals, representatives from the pharmaceutical industry and policy regulators.

Topics covered during the event included:

- The evolution of treatment of inhibitors
- Availability and limitations of treatment of inhibitors
- Inhibitor development: Current practices in data collection and post-marketing surveillance
- Survey of Inhibitors in Plasma-Product Exposed Toddlers (SIPPET) study
- Physician and industry perspective on whether to change or not to change treatment in previously untreated patients (PUPs)

## WORKSHOP ON TENDERS AND PROCUREMENT

*September 2016 – Baku, Azerbaijan*

In 2016, the EHC started a new three-year cycle of economics workshops, this time focusing on 'Tenders and Procurement.' The first workshop of this series was held in Baku, Azerbaijan and was aimed at EHC Members from outside the EU. The event was attended by participants from Azerbaijan, Bulgaria, Georgia, Kyrgyzstan, Latvia, Montenegro and Ukraine.

Experience shows that best overall outcomes are achieved when patient representatives and expert clinicians are involved in the national tenders for treatment. This inclusion not only leads to the purchase of safer and more efficacious product, but to a more cost-effective process as well. The objective of the workshop was to provide patients with the technical and practical knowledge on how to become more involved in their national tenders on coagulation factors concentrates. Participants were presented with a mixture of theoretical lectures and practical exercises and took part in a mock tender.

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

## EHC CONFERENCE

*October 2016 – Stavanger, Norway*

The 2016 EHC Conference brought together over 300 participants representing EHC NMOs and other patient organisations, medical doctors, physiotherapists and other health care providers within haemophilia and pharmaceutical companies. The variety of stakeholders assured for a comprehensive delivery of the latest scientific and medical updates on haemophilia and other rare bleeding disorders.

With interactive discussions, symposiums and debates, the conference was a platform for NMOs to gather and discuss matters of treatment, improving quality of life, new drugs and developments. A session of high interest was the debate between young participants and established leaders in the haemophilia field on specific issues in haemophilia care.

Topics covered during the conference included:

- The state of haemophilia care in Norway and other Nordic countries
- Updates on: dental care and von Willebrand treatment
- New developments in haemophilia care: gene therapy, bi-specific antibody, antithrombin and other novel technologies
- Inhibitors
- Presentation of patient-reported outcome data

Prior to the EHC Conference, the EHC held its General Assembly, which is composed of the NMOs. Among the taken decisions were election of the lay members of the SC and the unanimous approval of the EHC PARTNERS programme – a multi-stakeholder, multi-year project built to help PWH and their governments to gain significantly increased access to factor concentrates through a sustainable and step-wise approach. The work around it is set to start in the beginning of 2017.


29th EUROPEAN HAEMOPHILIA CONSORTIUM
ANNUAL CONFERENCE
Welcome
7-9 October | Stavanger | Norway
EHC
EHC
CONFERENCE
STAVANGER 2016
EHC
european haemophilia consortium

## WORKSHOP ON NEW TECHNOLOGIES IN HAEMOPHILIA CARE

*November 2016 – Berlin, Germany*

Haemophilia care yields best results when the treatment regime is individualised and the choice of treatment product is made by both patient and clinician. In continuation of providing its NMOs understanding and training on novel treatment developments, the EHC held its traditional Workshop on New Technologies in Haemophilia Care. The event gathered 50 participants from 15 countries including patients' representatives, health care professionals and regulators.

In addition to giving a detailed overview of new medicinal products that are being developed to treat haemophilia, the workshop fostered discussions on strategies for patients' access to novel treatment.

Covered topics included:

- Added value of extended half-life (EHL) products
- Laboratory monitoring
- Immunogenicity of current FVIII factor concentrates in PUPs and previously treated patients
- Gene therapy, biosimilars and inhibitor treatment
- Current challenges and benefits, effectiveness, and risk assessment in haemophilia
- Patient involvement in the assessment of new therapies in Germany

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

## ROUND TABLE ON PERSONALISED OUTCOME MEASURES

*November 2016 – Brussels, Belgium*

The last Round Table of the year was held in the European Parliament in Brussels and gathered over 50 participants. In light of the development of many novel technologies, representatives from patient organisations, healthcare professionals and the pharmaceutical industry gathered to discuss personalised outcome measures in haemophilia. The discussions concluded that outcome measures should be a key indicator of the real impact of novel technologies on the quality of life of patients.

Covered topics included:

- ▷ Significance of patient-reported outcome measures
- ▷ Outcome measures for older PWH
- ▷ Clinician and industry view on haemophilia outcome measures

*The EHC would like to thank Members of the European Parliament (MEPs) Mrs Norica Nicolai and Mrs Viorica Dăncilă for their support of the Round Table.*

## EUROPEAN INHIBITOR SUMMIT

*December 2016 – Barretstown, Ireland*

In December 2016, the EHC held its first Inhibitor Summit, an event bringing together people affected by bleeding disorders and inhibitors from across Europe. As part of the initiatives of the EHC Inhibitor Working Group (IWG) to address the needs of PWI, the summit consisted of workshops, peer-to-peer sessions, interactive presentations and above all, allowed for strong community-building.

Taking place in Ireland, it brought together more than 100 participants, speakers and facilitators from 26 European countries. This was a time and space where PWI, their families and caregivers could educate and empower themselves in order to improve their quality of life.

The programme of the summit included:

- Information sessions on inhibitors, current and future treatments, prophylaxis and surgery, differences in inhibitor treatment across Europe
- Workshops 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 an educational grant from Shire.*

## CREATION OF EHC ACTIVITY GRANT

In 2016, the EHC created a fund to support ad hoc activities for its NMOs. Consisting of €50,000 annually, the fund will be put at the disposal of EHC members to either initiate a project/programme, or to allow an already-initiated project/programme to continue.

The grant is meant to support projects that will benefit NMO members and directly support the improvement of the quality of life of people living with a rare bleeding disorder in their country.

# 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.**

### MoU WITH UNDP UKRAINE

In 2016, following increased efforts to support its Ukrainian NMO, the EHC signed a MoU with the UNDP Ukraine. This body supports the Ukrainian government in procuring coagulation factor concentrates for the Ukrainian paediatric haemophilia population. The EHC and UNDP Ukraine agreed to share information and best practices in the area of procurement of medicines for the treatment of haemophilia as well as information on medical practices and scientific development for the treatment of haemophilia.

The EHC is delighted to provide its support to the UNDP Ukraine to ensure and improve access to treatment for children with haemophilia in Ukraine.

Treatment level is unfortunately very low with an estimated 0.8 international units (IU) per capita in 2013, which lowered to 0.4 IU per capita in 2015.

# Data collection and sharing of best practices

The EHC understands that sound scientific evidence is the bedrock of its advocacy efforts. This is why the EHC conducts annual surveys and collects data on various aspects of access to treatment and care in Europe. The collected data is compiled, analysed and interpreted by the EHC; the results are frequently published in scientific journals such as *Haemophilia*. They have been 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), which in 2015 were turned into a resolution of the CoE.

The EHC also assures that its events programmes provide the opportunity for best practice exchange between NMOs. This allows for members to develop initiatives and better support their haemophilia communities.

## EHC YOUTH LEADERSHIP WORKSHOP 2016

*April 2016 – Barcelona, Spain*

For the third year in a row, the EHC welcomed fifteen young volunteers from haemophilia communities across Europe for a three-day workshop. With a mixture of presentations and role-playing, the workshop aims to train and equip the next generation of leaders with the tools to strengthen and continue the growth of their NMO.

The programme included:

- Sessions on communication: conflict resolution, decision making, negotiating skills, strategic planning
- Internal and external communication role-play exercises
- Strategic look at youth involvement in NMOs
- Panel discussions on industry and patient engagement

The EHC is committed to building a strong youth community across Europe and believes the workshops are a mutually motivating way to do so.

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

## EHC LEADERSHIP CONFERENCE

*June 2016 – Brussels, Belgium*

After the success of and interest in the Leadership Conference as a pre-EHC Conference event in 2015, the EHC decided to separate and expand its concept. In 2016, the first stand-alone Leadership Conference took place in Brussels and brought together more than 70 leaders, staff and youth volunteers from 23 NMOs. In addition to interactive exercises and workshops, the three-day event provided a space where NMOs can exchange information on best practices and ask for advice and support on ongoing national issues.

Covered areas of interest included:

- Communication strategies
- External relations with industry, policy-makers and regulators
- Negotiating skills
- NMO experiences and practices

*This conference was made possible thanks to educational grants from Sobi, Pfizer and Shire.*

## SURVEY ON HCV AND HAEMOPHILIA

To continue raising awareness about the needs of those in the haemophilia community who were infected with HCV, the EHC launched in 2016 a survey on HCV and haemophilia. The survey was sent to all EHC members in both English and Russian to identify the number of people with rare bleeding disorders contaminated with HCV, their clinical outcome as well as their access to HCV testing and treatment.

Data gathering and analysis occurred in 2017 and the findings of the study are expected to be published in third quarter of 2017. The EHC hopes that this data will support both national and European advocacy efforts to ensure access to novel direct-acting antivirals (DAAs) for PWH who were contaminated with HCV.


SOURCE
MESSAGE
RECEIVER


SAMSUNG


evian

# NMO liaison

In 2015 the EHC started a programme to allocate 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**<br>*Vice-President Finance* | **Radoslaw Kaczmarek**<br>*SC Member* | **Traci Marshall-Dowling**<br>*SC Member* | **Giuseppe Mazza**<br>*SC Member* | **Olivia Romero-Lux**<br>*SC Member* |
|---|---|---|---|---|
| Armenia | Czech Republic | Denmark | Albania | Austria |
| Azerbaijan | Estonia | Finland | Bosnia-Herzegovina | Belgium |
| Belarus | Hungary | Iceland | Croatia | France |
| Bulgaria | Latvia | Ireland | Cyprus | Germany |
| Georgia | Lithuania | Israel | Greece | Luxembourg |
| Kyrgyzstan | Moldova | Netherlands | Italy | Portugal |
| Russia | Poland | Norway | Macedonia | Slovenia |
| Turkey | Romania | Sweden | Montenegro | Spain |
| Ukraine | Slovakia | UK | Serbia | 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 factors. In 2016, the EHC took part in initiatives and organised several activities to meet its strategic objective of promoting access to treatment and comprehensive care.

## WORLD HAEMOPHILIA DAY: "TREATMENT FOR ALL, THE VISION OF ALL"

*April 2016 – Brussels, Belgium*

To mark World Haemophilia Day, the EHC held three separate events to highlight one urgent issue – access to treatment for all. For the rare bleeding disorders community, this concept has wider application as it includes various key areas that need immediate addressing - lack of access to treatment for HCV, haemophilia treatment disparities throughout countries and lack of unified care for an ageing haemophilia population.

### Event on HCV and haemophilia

HCV has been one of the leading causes of morbidity and mortality in the haemophilia population, a large part of which was infected through their government-provided treatment in the 1980s and early 1990s. Despite this, only a small proportion currently have access to DAAs, which have a 95% success rate of curing affected individuals. It is for this reason that the EHC has adopted a strategic focus on the eradication of HCV in the haemophilia community in the coming years.

World Haemophilia Day was an opportunity to raise awareness and call on European governments to establish national protocols that assure haemophilia patients access to HCV treatment.

Topics covered during the event were:

- HCV: A call for action in Europe
- Eradication of HCV in haemophilia
- Current and upcoming HCV treatments: Challenges and opportunities
- HCV treatment in resource-limited countries
- Eradicating HCV: The Portuguese experience

### Screening of the documentary 'Haemophilia Stories'

The ongoing disparities in access to treatment throughout Europe were illustrated through the screening of the documentary 'Haemophilia Stories,' a project the EHC worked on together with director/producer Goran Kapetanovic. The film took audiences on a journey across five European countries, highlighting the different health outcomes for PWH based on their geographical location. At the moment, there is still a 17-fold difference in terms of access to haemophilia treatment between some countries with the best and worst access in Europe.

*The EHC would like to thank Roche and Sobi for making the World Haemophilia Day events possible through educational grants.*

## Round Table of Stakeholders on Ageing and Haemophilia

With no effective treatment until the 1960s, patients born with severe haemophilia before that time had a life-expectancy of less than twenty years. The introduction of coagulation factor concentrates changed this grim statistic and today, health care professionals are for the first time confronted with a large ageing haemophilia patient population. This raises many medical questions on how to manage haemophilia with comorbidities arising both from older age, such as cardiovascular or metabolic diseases and cancer; and haemophilia-related complications, such as arthropaty, inhibitors and viral infections due to the 1980s-90s treatment product contamination with HCV and HIV.

Given these pressing issues, the EHC organised a Round Table of Stakeholders to look at treatment protocols in use and establish which health care services are needed for these patients.

Topics covered at the Round Table included:

- Treatment protocols and organisation of care
- Treatment of cardiac problems in older PWH
- Physiotherapy for older PWH
- Personal experiences
- NMO activities for an ageing haemophilia population

The discussions clearly displayed the need for a multidisciplinary approach, as a series of medical experts need to be involved in the care of the ageing PWH. Better information, education and coordination is needed for both health care professionals and patients. The EHC will continue with its initiations to facilitate such exchange of knowledge.

*We would like to thank MEPs Dr Miroslav Mikolášik, Mrs Karin Kadenback, Mr Heinz Becker and Mrs Sirpa Pietikäinen who supported, chaired and spoke at these events.*

## ROUND TABLE ON HCV AND HAEMOPHILIA

*June 2016 – Brussels, Belgium*

As part of the EHC's 2016 focus on improving the access of PWH to new and highly-effective HCV treatment, the organisation held an extra-ordinary Round Table on the topic in the European Parliament (in addition to the normal set of three Round Tables per year).

In many European countries, access to the DAAs is being prioritised according to clinical need, which is usually defined by the severity of progression of liver disease. In this scenario, PWH may currently have to wait until they have cirrhosis or significant fibrosis before they are offered treatment.

The Round Table on HCV and Haemophilia brought together patients, scientists, physicians, policy makers and industry to discuss the challenge of access to HCV treatment.

Topics addressed during the Round Table included:

- HCV and Haemophilia
- HCV: Why treat?
- Current and future liver treatment options
- Eradication of HCV: Patients' perspective
- National HCV programmes: A European approach to eliminating HCV
- Examples of Ireland and Portugal national plan for HCV eradication

The Round Table was one of several EHC initiatives to raise awareness amongst policy makers who work on rare bleeding disorders about the need for an effective and fast HCV eradication policy.

*The EHC would like to thank MEP Dr Cristian-Silvu Busoi for chairing the event and MEP Dr Andrey Kovatchev for his support of it.*


pe
pe
ment européen
Andrey KOVATCHEV
MEP
Cristian BUŞOI
MEP
Brian O'MAHONY
EHC President

## EHC-EAHAD MEETING OF CLINICIANS: DEVELOPING GUIDELINES FOR INHIBITOR MANAGEMENT

*July 2016 – Brussels, Belgium*

Though regularly working together, in 2016 the EHC and the European Association for Haemophilia and Allied Disorders (EAHAD) held their first official and high-level meeting on the topic of inhibitors. The two organisations and other participants brought into discussion the aspects, challenges and needs of people with rare bleeding disorders and inhibitors. To address these issues, the meeting highlighted the need to develop European principles on the management of inhibitors.

The EHC and EAHAD will further meet to expand their activities and to prepare recommendations for inhibitor care.

ITI protocols used*

| ITI protocols | Children | Adults |
|---|---|---|
| High dose | 30% | 24% |
| Bonn | 15% | 12% |
| Malmö | 7% | 8% |
| Low dose | 5% | 12% |
| Depending on titre/ individualised | 28% | 28% |
| Other: | 15% | 16% |
| ObsITI | 2 | 0 |
| BCSH guidelines | 1 | 1 |
| UKHDO recommendations | 2 | 0 |
| Van Creveld model | 1 | 1 |
| 100 U/kg/12-24h | 0 | 2 |

*With or without immunosuppression

## EHC ATTENDANCE OF WILDBAD KREUTH IV INITIATIVE

In May 2016, representatives from the EHC attended the meeting organised by the EDQM, University of Munich and the CoE on the optimal use of coagulation factors and platelets (also known as the Wildbad Kreuth IV initative). The meeting was attended by regulators, clinicians, patient representatives and representatives from the pharmaceutical industry from all over Europe.

The meeting was held to discuss, amongst others, challenges in the clinical use of clotting factors, inhibitor development and access to treatment. It resulted in a set of 12 recommendations for the treatment of haemophilia, which were published in the scientific journal *Haemophilia* in April 2017. These recommendations are currently being reviewed by the CoE to be potentially adopted as a resolution.

## RECOMMENDATIONS

**1.** Hospitals providing clinical care for people with haemophilia and related disorders are strongly recommended to seek formal designation as either EHCCC or EHTC. (Access to comprehensive care and replacement therapy should be equitable in all parts of a country).

**2.** There should be agreed national protocols or guidelines on management of the ageing patient with haemophilia. Treatment centres are encouraged to include an appropriate general physician in the comprehensive care team.

**3.** The minimum consumption of factor VIII concentrate in any country should be 4 iu/capita of general population. (Data expressed as units/ severe patient should also be collected in parallel in future.)

**4.** The minimum consumption of FIX concentrate in a country should be 0.5 iu/ per capita of general population.

**5.** Treatment for hepatitis C with direct-acting antiviral agents should be provided to all people with haemophilia on a high priority basis.

**6.** Genotype analysis must be offered to all patients with severe haemophilia and the results used to identify carriers in the wider family.

**7.** People with inhibitors should have access to immune tolerance.

**8.** People with inhibitors should also have access to elective surgery at a specialist centre with relevant experience.

**9.** National or regional tenders for factor concentrates are encouraged and should always include both haemophilia clinicians and national haemophilia patient representatives.

**10.** Outcome data including health related quality of life should be collected with appropriate study design, e.g. annualised bleed rates (ABR), mortality, joint score and time off education or employment.

**11.** Treatment with extended half-life factors should be individualized and protection against bleeding should be improved by increasing trough levels.

**12.** There is increasing evidence that the incidence of inhibitors amongst previously–untreated patients (PUPs) varies between products. Steps should be taken to understand and minimise this risk. (Patients, or their parents, should be involved in discussions related to product choice.)

# 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.

## Patient Organisations

### WORLD FEDERATION OF HEMOPHILIA (WFH)

WFH
WORLD FEDERATION OF HEMOPHILIA

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 SC and in turn, the EHC President or his/her designee is invited annually to attend the 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 conferences and coordinating joint positions at European, international and national levels when necessary and appropriate.

This year, EHC representatives attended the 2016 WFH World Congress held in Orlando, USA. This was an opportunity for the EHC representatives to meet with the global haemophilia community and be informed of issues faced by the haemophilia community and initiatives happening in other world regions.

To improve its visibility and promote its work the EHC held an exhibition stand, as well as a meeting with many of its NMOs that were also present to provide an overview of its current work and priorities. This was in line with the MoU between the EHC and WFH.

### EUROPEAN PATIENTS' FORUM (EPF)

EPF is an umbrella patient organisation representing chronic and lifelong conditions across Europe. In 2016, the EHC continued to take active part in their work on access to healthcare 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, GBS/CIDP Foundation International, HAEI, the WFH, APLUS and the International Patient Organisation for Primary Immunodeficiency. PLUS meets every year to discuss pressing topics concerning plasma products and their impact on patients' health. Mr Brian O'Mahony, EHC President, was elected to the PLUS SC in 2015.

### EURORDIS

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 or rare diseases. In 2016, the EHC was an active participant of EURORDIS' Rare Disease Day activities and the EURORDIS members' meeting. The EHC also worked closely with EURORDIS within the European Commission's Expert Group for Rare Diseases.

## 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 share a MoU, which formally sets the terms in which both organisations interact. The EHC and EAHAD speak at each other's conferences 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 2016, the two organisations held a joint meeting to discuss quality and standards of inhibitor care in Europe and to begin work on developing guidelines for the management of inhibitors (see pg. 38)

# 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 rare 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 when forming policies that affect the lives of people with these conditions in Europe.

## Informal MEP group ‘Friends of Haemophilia’

The European Parliament is an important player in developing European legislation and policies and as such, it represents the voice of the people. For many years, the EHC has been reaching out to Members of the MEPs to provide information about bleeding disorders and the needs of those living with these conditions. In 2014, the EHC formed a small informal group of MEPs ‘Friends of Haemophilia,’ chaired by Mrs Nessa Childers (Ireland) and composed of Dr Paul Rübig (Austria), Dr Miroslav Mikolášik (Slovakia) and Dr Cristian Bușoi (Romania). This group will be formalised in 2017.

## European Commission expert group on rare diseases

The EHC is one of the patient organisations representing patients' interests within the European Commission expert group on rare diseases. This committee works together with various stakeholders such as regulators, Member State representatives, European Commission officials and others to develop policies and to support people with rare diseases and to support the work of the European Commission and the Member States in this regard.

In 2016, the group finalised all documents needed to establish the European Reference Networks (ERNs) – virtual networks that allow for health care professionals across Europe to collaborate and exchange knowledge on rare and complex diseases. In efforts to ensure that patient organisations participate in ERN decision-making processes, 'European patient advocacy groups' (ePAG) were created for each ERN disease grouping. Amanda Bok, EHC CEO, was one of five patient organisation representatives to be elected on the ePAG of EuroBloodNet – the ERN that is relevant to haemophilia.

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

The EDQM, a CoE Directorate, is responsible for protecting public health by enabling the development, supporting the implementation and monitoring the application of quality standards for safe medicines and their safe use. It is one of the main partners of the Wildbad Kreuth initiatives (see pg. 38) and the EHC works with them closely on the development and promotion of standards and recommendations.

## European Medicines Agency (EMA)

The EHC is one of the patient organisations recognised by the EMA and in 2016 EHC representatives took part in various events organised by this agency. In addition, Dr Günter Auerswald continues to sit on the EMA Paediatric Committee as a representative on behalf of the EHC.

# 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 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) have to adhere to a code of conduct and declare conflicts of interest. 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 grows, 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 SC 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's 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 order to ensure the clear and ethical behaviour of all staff and volunteers, the EHC requires everyone who collaborates with the organisation (paid or unpaid) to complete and sign codes of conduct as well as declaration of interest forms.

## Ensure constructive ongoing partnership and engagement between the EHC SC and the MAG

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 – composed of clinicians. To this end, each body has a dedicated Terms of Reference and has joint meetings to ensure maximum dialogue, mutual engagement and alignment on key issues.

## Ensure adequate staff and financial resources to implement the strategic plan

In 2016, the EHC continued to expand its activities and focus. To be able to do so, the organisation hired an Office Assistant who helps to support the increasing workload and initiatives.

The closing of 2016 with more than €425,000 in positive income over expenditures allowed the EHC to strengthen its operational reserves.

## Develop and implement clear plan for diversification of funding

The EHC continued to look at opportunities to diversify its income and to ensure the independence and long-term sustainability of the EHC as a patient organisation.

# Structure and governance

# Steering Committee (SC)

In October, the EHC General Assembly elected the lay members of the SC for a new three-year term. The EHC was delighted to see both Radoslaw Kacmarek (Polish NMO) and Olivia Romero-Lux (French NMO) re-elected for their second terms and welcomed Miguel Crato (Portuguese NMO) and Naja Skouw-Rasmussen (Danish NMO) as new, first-term members. The organisation is also pleased to announce that Giuseppe Mazza (Italian NMO) was co-opted by the new SC at its first meeting in December.

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
- Alain Weill, Ex-Officio, WFH President
- Amanda Bok, Ex-Officio, EHC CEO

*The EHC wants to specially thank the outgoing SC members Traci Marshall-Dowling (Irish NMO) and Michael van der Linde (Dutch NMO). We are grateful for their invaluable contribution to the EHC and for their continued involvement in the EHC Youth Committee.*

# 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

# EHC Committees

## DATA AND ECONOMICS COMMITTEE (DEC)

This year, the EHC was delighted to welcome a new DEC member.  This is an EHC committee that advises and provides expertise to the EHC SC on matters related to health economics and data collection. Dr Alec Miners, Senior Lecturer in Health Economics at the London School of Hygiene and Tropical Medicine joins other medical experts in the hopes that through the production of sound scientific data, the EHC will be able to provide its NMOs with the right tools to further conduct advocacy work in their respective countries. The DEC is composed of:

| | |
|---|---|
| **Chair – Declan Noone** | Irish NMO |
| Amanda Bok | EHC staff |
| Mariëtte Driessens | Dutch NMO |
| Michael Makris | MAG member / University of Sheffield |
| Giuseppe Mazza | SC member / Italian NMO |
| Jamie O'Hara | UK NMO |
| Brian O'Mahony | EHC President / Irish NMO |
| Frits Rosendaal | Leiden University |
| Alec Miners | London School of Hygiene and Tropical Medecine |
| Laura Savini | EHC staff |
| Uwe Schlenkrich | German NMO |
| Keith Tolley | Tolley Health Economics |

## 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 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 staff |
| Olivia Romero-Lux | EHC SC Member / French NMO |
| Thomas Sannié | French NMO |
| Ivan Sebest | Slovakian NMO |
| Traci Marshall-Dowling | Irish NMO |

## INHIBITOR WORKING GROUP (IWG)

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

It is composed of:

| | |
|---|---|
| Amanda Bok | EHC staff |
| Christina Burgess | UK NMO |
| Miguel Crato | Portuguese NMO |
| Paul Giangrande | EHC MAG Chair/ Oxford University |
| Carl Fredrik Gustafsson | Swedish NMO |
| Kristine Jansone | EHC staff |
| Mirko Jokic | Serbian NMO |
| Hannele Kareranta | Finnish NMO |
| Radoslaw Kaczmarek | EHC SC Member/ Polish NMO |
| Brian O'Mahony | EHC President/ Irish NMO |
| Oleksandra Stasyshin | Ukrainian NMO |

## 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 |
| Jo Eerens | EHC staff |
| Radoslaw Kaczmarek | SC Member/ Polish NMO |
| Olivia Romero-Lux | SC member / French NMO |
| Laura Savini | EHC staff |

*The EHC wishes to think all NMOs and external contributors who submitted material to be published in the newsletter.*

## Volunteers

COVINGTON

EHC volunteers contribute with invaluable work, support and advancement of EHC activities and initiatives. We most sincerely thank them for their time, efforts and passion!

In 2016, the EHC bid farewell to Ms Katja Kirchkevitch, a volunteer from the EHC Belarussian NMO who supported the EHC for many years with the translation of its communication and administrative material from English into Russian. Due to increased professional engagements, Ms Kirchkevitch was no longer able to devote the necessary time to volunteer for the EHC. The EHC thanks Ms Kirchkevitch for her work and wishes her all the best in her professional endeavours.

The EHC was nonetheless delighted to welcome Mr Sergiy Shemet, Board Member of the EHC Ukrainian NMO, as a replacement for Ms Kirchkevitch. Besides ensuring that critical administrative, communication and policy information is correctly translated into Russian, Mr Shemet has also provided support in translating the full EHC newsletters from English to Russian. Additionally, Mr Shemet has been advising EHC staff on how to ensure that the EHC communication strategy and material are more accessible to NMOs using primarily Russian as a vehicular language. The EHC welcomes and thanks Mr Shemet for his invaluable support.

In addition to the volunteers mentioned above, we also express our gratitude to those who provided ad hoc support to the EHC in 2016:

- Steffen Hartwig for his IT support and services
- Covington for their pro-bono legal support

The EHC would also like to extend its thank you to the local volunteers that ensure a smooth running of its Annual Conference and other events.

# Staff

To support its growing work, in 2016 the EHC expanded its team and welcomed Mrs Saskia Pfeyffer as an office assistant.

The EHC office is based in Brussels with staff members:

- Amanda Bok, EHC CEO
- Jo Eerens, EHC Office Manager
- Kristine Jansone, EHC Inhibitor Programme Officer
- Laura Savini, EHC Communications and Public Policy Officer
- Saskia Pfeyffer, EHC Office Assistant

# Service Providers

The EHC would like to thank the Conference Organisers for their help in the logistical organisation of the EHC events; Toast for their work on visual and print communication tools; RPP Group for their support of our EU-level work; BDO for their accounting services; Stéphane Rosier, our external auditor; Ethical Properties for making it possible for NGOs to be in the European Quarter; Goran Kapetanovic for capturing the reality of those living with bleeding disorders in Europe; Luka Pilipovic and Elliot Laub for visually documenting our events and last, but not least, Mrs Olga Miniuk and Mrs Julia Krivkina for their tireless work and dedication towards ensuring our Russian speaking members can fully participate in our conferences and workshops.

# Financial
## Report

# Word from the Vice-President Finance

The EHC has been growing from strength to strength and we were delighted and grateful to see this trajectory continue in 2016, enabled and supported by our strong financial and non-financial partnerships in Europe.

In 2016 our organisation saw a 10% growth in EHC Conference income, 20% growth in unpaid volunteer contributions and 60% growth in sponsorship base over the previous year, thereby cumulatively resulting in a near 20% growth of our overall combined income (both financial and in-kind volunteer contributions) between 2015 and 2016.
This growing financial and non-financial base enabled us not only to sustain but also to deepen and extend on all of our previous areas of work as outlined in this Annual Report and as reflected in the 40% growth of our expenditures in 2016 as compared to 2015.
Having in 2015 met our one-year operational reserve target, we chose to finance the above increased organisational output and therefore our accounts reflect a 15% reduction in financial surplus in 2016 from what they were in 2015. We nonetheless still closed 2016 with more than €425,000 in positive income over expenditure, which we allocated straight back into our operational reserves and thereby left our organisation in a strong position from which to start 2017.
In line with our continued commitment to transparency and external oversight, our 2016 accounts were independently audited under Belgian Audit Standards and the final accounts and audit report are available in the next pages.

# Report of the auditor

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 2016

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 2016, prepared in accordance with the financial-reporting framework applicable in Belgium, which show a balance sheet total of € 1.896.314 and a positive result for the year of € 426.733.

*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*

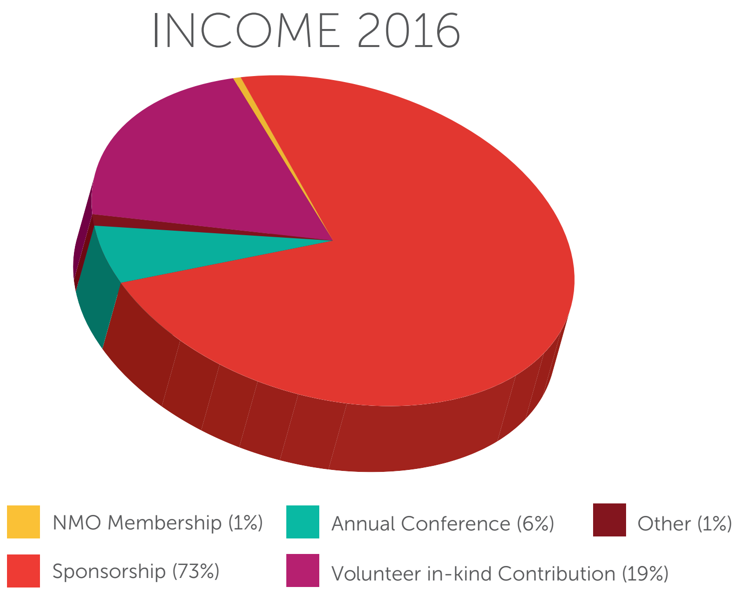
In our opinion, the annual accounts of the AISBL’s give a true and fair view of the association’s equity and financial position as at 31 December 2016, and of the results of its operations for the year then ended, in accordance with the financial-reporting framework applicable in Belgium.

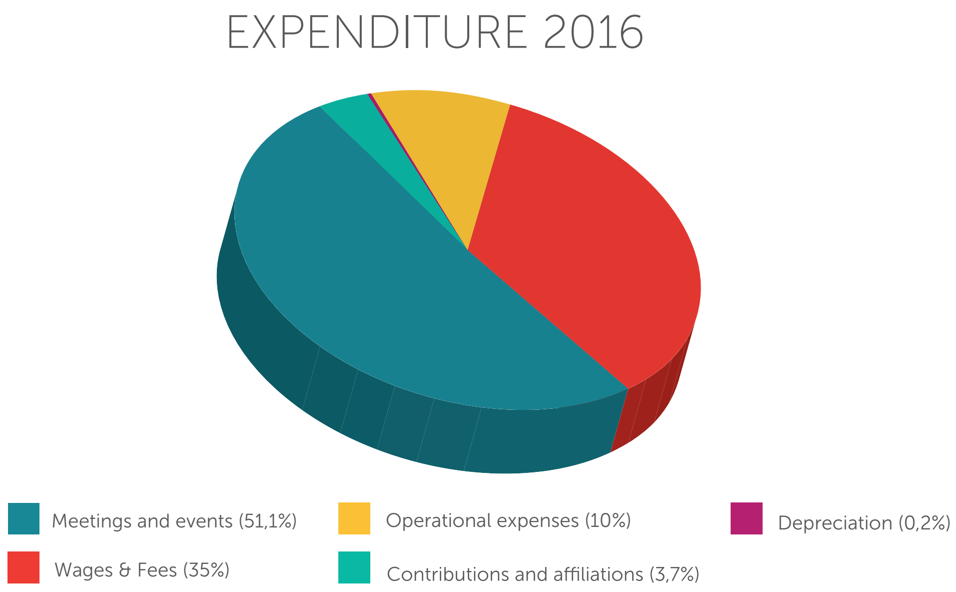
Limal, August 16$^{\&)}$2017

Rosier & Co
Independent auditor's

represented by
Stéphane Rosier
Certified Public Auditor

# Income and expenditure

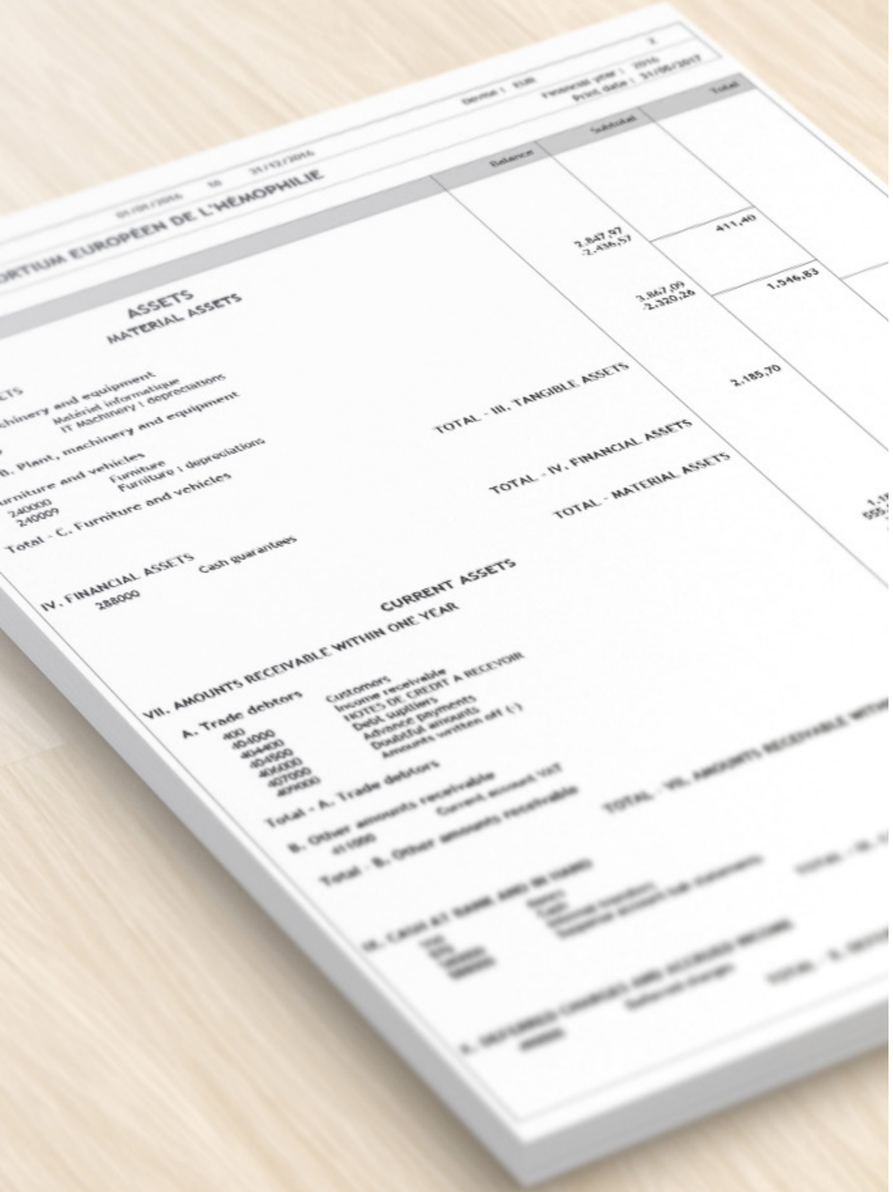


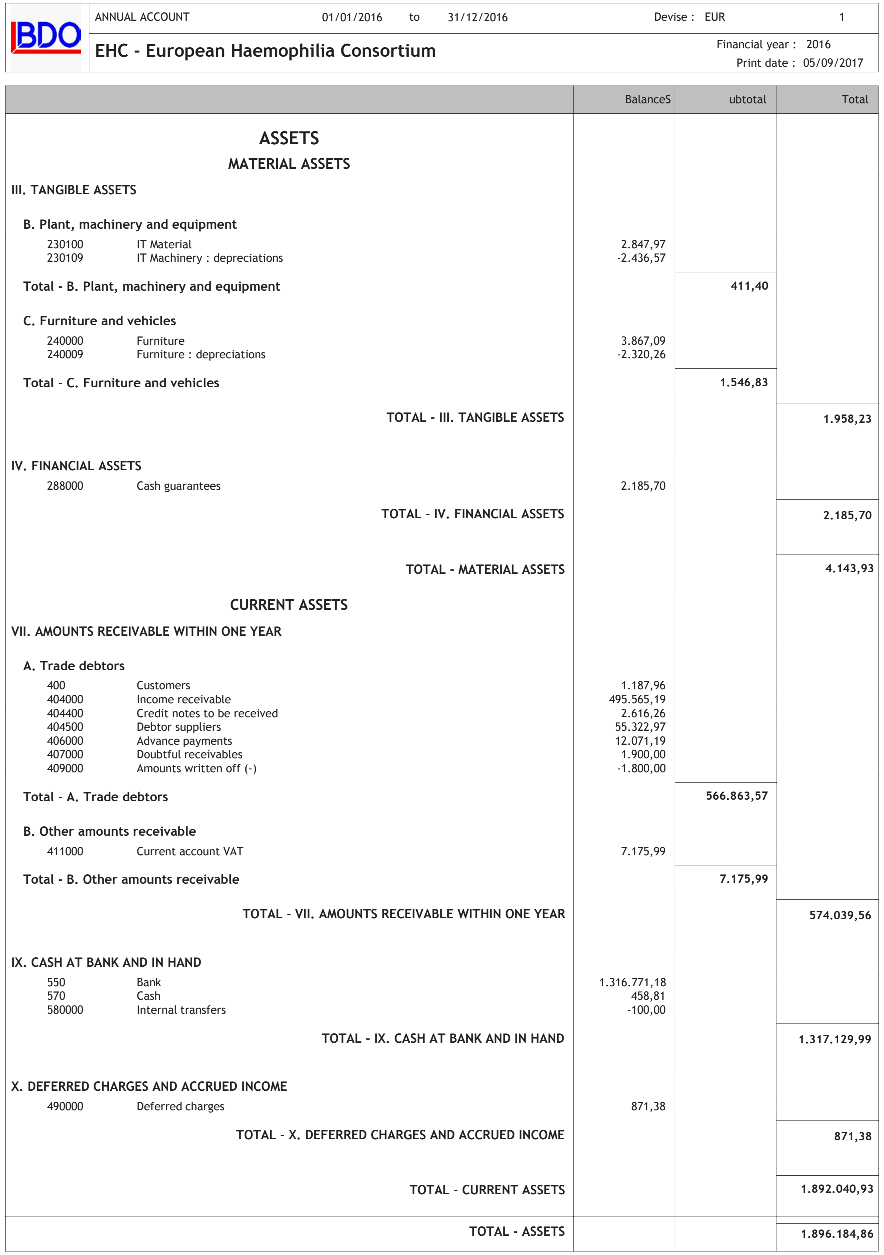


| **INCOME** | FINALS (€) |
|---|---|
| NMO Membership | 10,800 |
| Sponsorship | 1,121,348 |
| Annual Conference | 85,528 |
| Other | 15,145 |
| SUB-TOTAL INCOME | 1,232,821 |
| Volunteer in-kind Contribution | 296,700 |
| TOTAL INCOME | 1,529,521 |

| **EXPENDITURE** | FINALS (€) |
|---|---|
| Operational expenses | 80,236 |
| Wages and fees | 282,201 |
| Meetings and events | 411,426 |
| Contributions and affiliations | 30,453 |
| Depreciation | 1,533 |
| TOTAL EXPENDITURE | 805,849 |

# Balance Sheet





| ANNUAL ACCOUNT | 01/01/2016 | to | 31/12/2016 | Devise : EUR | 1 |
|---|---|---|---|---|---|
| EHC - European Haemophilia Consortium | | | | Financial year : 2016<br>Print date : 05/09/2017 | |

| | | BalanceS | ubtotal | Total |
|---|---|---|---|---|
| **ASSETS** | | | | |
| **MATERIAL ASSETS** | | | | |
| **III. TANGIBLE ASSETS** | | | | |
| **B. Plant, machinery and equipment** | | | | |
| 230100 | IT Material | 2.847,97 | | |
| 230109 | IT Machinery : depreciations | -2.436,57 | | |
| **Total - B. Plant, machinery and equipment** | | | 411,40 | |
| **C. Furniture and vehicles** | | | | |
| 240000 | Furniture | 3.867,09 | | |
| 240009 | Furniture : depreciations | -2.320,26 | | |
| **Total - C. Furniture and vehicles** | | | 1.546,83 | |
| | **TOTAL - III. TANGIBLE ASSETS** | | | 1.958,23 |
| **IV. FINANCIAL ASSETS** | | | | |
| 288000 | Cash guarantees | 2.185,70 | | |
| | **TOTAL - IV. FINANCIAL ASSETS** | | | 2.185,70 |
| | **TOTAL - MATERIAL ASSETS** | | | 4.143,93 |
| **CURRENT ASSETS** | | | | |
| **VII. AMOUNTS RECEIVABLE WITHIN ONE YEAR** | | | | |
| **A. Trade debtors** | | | | |
| 400 | Customers | 1.187,96 | | |
| 404000 | Income receivable | 495.565,19 | | |
| 404400 | Credit notes to be received | 2.616,26 | | |
| 404500 | Debtor suppliers | 55.322,97 | | |
| 406000 | Advance payments | 12.071,19 | | |
| 407000 | Doubtful receivables | 1.900,00 | | |
| 409000 | Amounts written off (-) | -1.800,00 | | |
| **Total - A. Trade debtors** | | | 566.863,57 | |
| **B. Other amounts receivable** | | | | |
| 411000 | Current account VAT | 7.175,99 | | |
| **Total - B. Other amounts receivable** | | | 7.175,99 | |
| | **TOTAL - VII. AMOUNTS RECEIVABLE WITHIN ONE YEAR** | | | 574.039,56 |
| **IX. CASH AT BANK AND IN HAND** | | | | |
| 550 | Bank | 1.316.771,18 | | |
| 570 | Cash | 458,81 | | |
| 580000 | Internal transfers | -100,00 | | |
| | **TOTAL - IX. CASH AT BANK AND IN HAND** | | | 1.317.129,99 |
| **X. DEFERRED CHARGES AND ACCRUED INCOME** | | | | |
| 490000 | Deferred charges | 871,38 | | |
| | **TOTAL - X. DEFERRED CHARGES AND ACCRUED INCOME** | | | 871,38 |
| | **TOTAL - CURRENT ASSETS** | | | 1.892.040,93 |
| | **TOTAL - ASSETS** | | | 1.896.184,86 |

ANNUAL ACCOUNT 01/01/2016 to 31/12/2016 Devise : EUR 3

**EHC - European Haemophilia Consortium**

Financial year : 2016
Print date : 05/09/2017

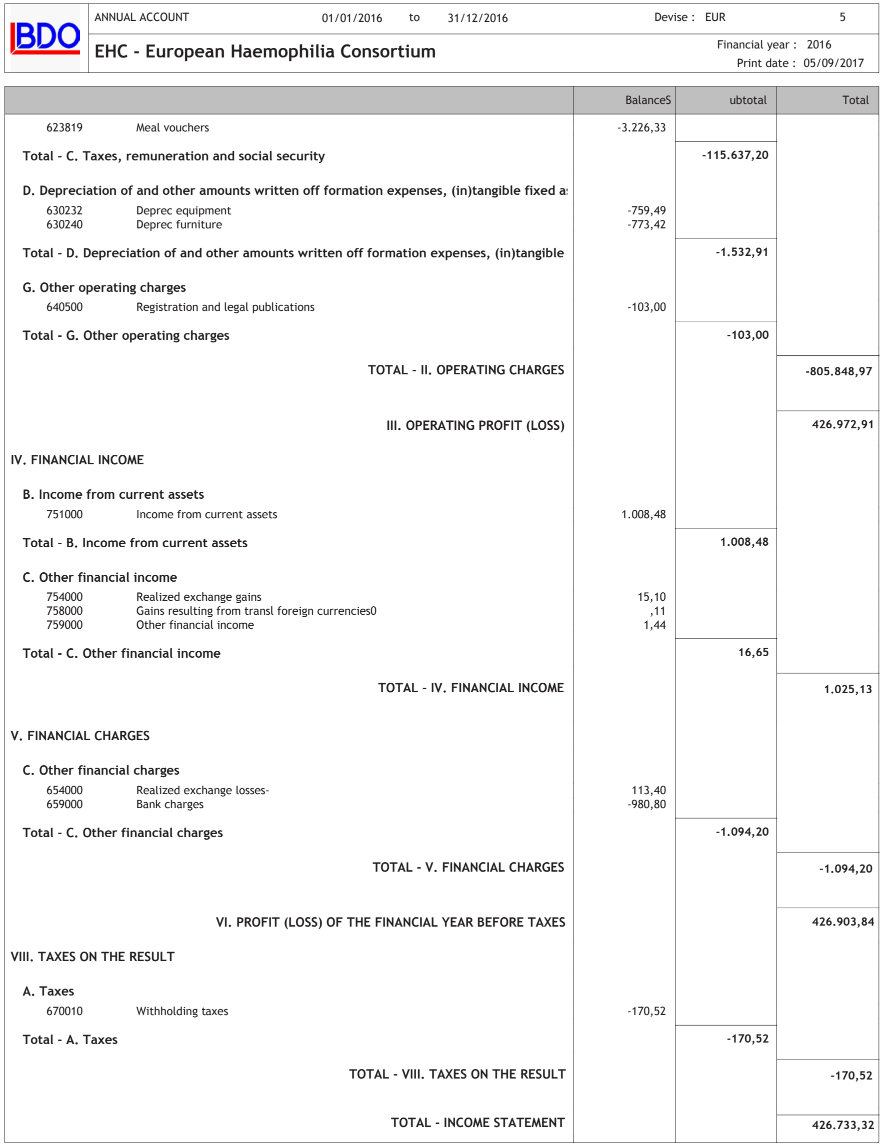
| | | BalanceS | ubtotal | Total |
|---|---|---|---|---|
| **LIABILITIES** | | | | |
| **CAPITAL AND RESERVES** | | | | |
| **I. CAPITAL** | | | | |
| **A. Issued capital** | | | | |
| 100000 | Issued capital | 9.419,96 | | |
| **Total - A. Issued capital** | | | **9.419,96** | |
| **TOTAL - I. CAPITAL** | | | | **9.419,96** |
| **V. PROFIT CARRIED FORWARD** | | | | |
| 140000 | Accumulated profits | 856.157,16 | | |
| **TOTAL - V. PROFIT CARRIED FORWARD** | | | | **856.157,16** |
| **TOTAL - CAPITAL AND RESERVES** | | | | **865.577,12** |
| **CREDITORS** | | | | |
| **IX. AMOUNTS PAYABLE WITHIN ONE YEAR** | | | | |
| **C. Trade debts** | | | | |
| 1. Suppliers | | | | |
| 440 | Suppliers | 40.427,37 | | |
| 444000 | Invoices to be received | 71.008,48 | | |
| Total - 1. Suppliers | | 111.435,85 | | |
| **Total - C. Trade debts** | | | **111.435,85** | |
| **E. Taxes, remuneration and social security** | | | | |
| 2. Remuneration and social security | | | | |
| 454000 | Social security contributions | -286,67 | | |
| 456000 | Holiday pay | 13.765,40 | | |
| Total - 2. Remuneration and social security | | 13.478,73 | | |
| **Total - E. Taxes, remuneration and social security** | | | **13.478,73** | |
| **F. Other amounts payable** | | | | |
| 489000 | Sundry amounts payable- | 130,00 | | |
| **Total - F. Other amounts payable** | | | **-130,00** | |
| **TOTAL - IX. AMOUNTS PAYABLE WITHIN ONE YEAR** | | | | **124.784,58** |
| **X. ACCRUED CHARGES AND DEFERRED INCOME** | | | | |
| 492000 | Accrued charges | 103,00 | | |
| **TOTAL - X. ACCRUED CHARGES AND DEFERRED INCOME** | | | | **103,00** |
| **TOTAL - CREDITORS** | | | | **124.887,58** |
| **TOTAL - LIABILITIES** | | | | **990.464,70** |

ANNUAL ACCOUNT 01/01/2016 to 31/12/2016 Devise : EUR 4

**EHC - European Haemophilia Consortium**

Financial year : 2016
Print date : 05/09/2017

| | | BalanceS | ubtotal | Total |
|---|---|---|---|---|
| **INCOME STATEMENT** | | | | |
| **I. OPERATING INCOME** | | | | |
| **A. Turnover** | | | | |
| 700000 | NMO MEMBERSHIP FEES | 10.800,00 | | |
| 700100 | MEMBERSHIP CORPORATE PROGRAM | 162.250,00 | | |
| 700200 | MEMBERSHIP ROUND TABLE | 202.500,00 | | |
| 700300 | SPONSORSHIP WORKSHOP | 360.000,00 | | |
| 700400 | SPONSORSHIP WHD | 20.000,00 | | |
| 700500 | EHC CONFERENCE | 85.528,19 | | |
| 700700 | OTHER GRANTS | 376.598,23 | | |
| **Total - A. Turnover** | | | **1.217.676,42** | |
| **D. Other operating income** | | | | |
| 743200 | RECHARGE OF EXPENSES | 7.282,76 | | |
| 743900 | SOCIAL WITHHOLDING TAXES REFUND | 355,01 | | |
| 744000 | OTHER OPERATING INCOME | 7.507,69 | | |
| **Total - D. Other operating income** | | | **15.145,46** | |
| **TOTAL - I. OPERATING INCOME** | | | | **1.232.821,88** |
| **II. OPERATING CHARGES** | | | | |
| **B. Services and other goods** | | | | |
| 610000 | Rent | -22.619,07 | | |
| 610100 | Rental charges | -2.156,55 | | |
| 612200 | Fuel vehicles | -28,00 | | |
| 612300 | Telephone, internet | -3.220,66 | | |
| 612310 | Website | -1.314,30 | | |
| 6123101 | Film | -800,00 | | |
| 612400 | Postal charges | -425,74 | | |
| 612600 | Office supplies | -16.761,54 | | |
| 612900 | Expenses to be reimboursed | -10.191,38 | | |
| 613100 | Fire insurances | -238,31 | | |
| 613150 | RC insurance | -624,28 | | |
| 613160 | Insurance:civil responsibility- | 174,23 | | |
| 613170O | ther insurances | -118,82 | | |
| 613200 | Translation fees | -1.199,25 | | |
| 613201 | Conference Organisers fees | -257.355,06 | | |
| 613202 | Consultancy | -66.208,56 | | |
| 613203 | Public Affairs fees | -58.231,19 | | |
| 613220 | Fee chartered accountants- | 19.835,50 | | |
| 613230 | Fee tax auditor | -1.500,00 | | |
| 613250 | Fee social secretary | -1.510,90 | | |
| 613270E | xpenses of meal vouchers- | 337,14 | | |
| 613290 | Consultancy Fees | -98.507,10 | | |
| 613300 | Contributions and affiliations | -14.453,05 | | |
| 613310 | Legal publications | -124,63 | | |
| 613400 | Accomodation costs | -26.263,68 | | |
| 613410 | Travel | -33.566,82 | | |
| 613420 | Restaurant | -7.130,76 | | |
| 613430 | Public transport | -2.180,76 | | |
| 613431 | Mileage allowance | -329,00 | | |
| 613440 | Parking | -1.429,58 | | |
| 613450 | Taxi costs | -7.033,01 | | |
| 613460 | Reception costs | -15.741,09 | | |
| 614100 | Conferences and Events | -661,66 | | |
| 614400 | Business Gifts | -255,74 | | |
| 614500 | Medals | -48,50 | | |
| 617200 | Donations | -16.000,00 | | |
| **Total - B. Services and other goods** | | | **-688.575,86** | |
| **C. Taxes, remuneration and social security** | | | | |
| 620200 | Salaried staff | -82.266,13 | | |
| 620400 | Holiday pay | -6.708,82 | | |
| 621000 | Employer's social security contributions | -20.874,98 | | |
| 623000 | Other personnel | -1.560,42 | | |
| 623100 | Medical services | -250,22 | | |
| 623200 | Catering | -750,30 | | |



BDO

ANNUAL ACCOUNT 01/01/2016 to 31/12/2016 Devise : EUR 5

**EHC - European Haemophilia Consortium**

Financial year : 2016
Print date : 05/09/2017

| | | BalanceS | ubtotal | Total |
|---|---|---|---|---|
| 623819 | Meal vouchers | -3.226,33 | | |
| **Total - C. Taxes, remuneration and social security** | | | -115.637,20 | |
| **D. Depreciation of and other amounts written off formation expenses, (in)tangible fixed a** | | | | |
| 630232 | Deprec equipment | -759,49 | | |
| 630240 | Deprec furniture | -773,42 | | |
| **Total - D. Depreciation of and other amounts written off formation expenses, (in)tangible** | | | -1.532,91 | |
| **G. Other operating charges** | | | | |
| 640500 | Registration and legal publications | -103,00 | | |
| **Total - G. Other operating charges** | | | -103,00 | |
| **TOTAL - II. OPERATING CHARGES** | | | | -805.848,97 |
| **III. OPERATING PROFIT (LOSS)** | | | | 426.972,91 |
| **IV. FINANCIAL INCOME** | | | | |
| **B. Income from current assets** | | | | |
| 751000 | Income from current assets | 1.008,48 | | |
| **Total - B. Income from current assets** | | | 1.008,48 | |
| **C. Other financial income** | | | | |
| 754000 | Realized exchange gains | 15,10 | | |
| 758000 | Gains resulting from transl foreign currencies0 | ,11 | | |
| 759000 | Other financial income | 1,44 | | |
| **Total - C. Other financial income** | | | 16,65 | |
| **TOTAL - IV. FINANCIAL INCOME** | | | | 1.025,13 |
| **V. FINANCIAL CHARGES** | | | | |
| **C. Other financial charges** | | | | |
| 654000 | Realized exchange losses- | 113,40 | | |
| 659000 | Bank charges | -980,80 | | |
| **Total - C. Other financial charges** | | | -1.094,20 | |
| **TOTAL - V. FINANCIAL CHARGES** | | | | -1.094,20 |
| **VI. PROFIT (LOSS) OF THE FINANCIAL YEAR BEFORE TAXES** | | | | 426.903,84 |
| **VIII. TAXES ON THE RESULT** | | | | |
| **A. Taxes** | | | | |
| 670010 | Withholding taxes | -170,52 | | |
| **Total - A. Taxes** | | | -170,52 | |
| **TOTAL - VIII. TAXES ON THE RESULT** | | | | -170,52 |
| **TOTAL - INCOME STATEMENT** | | | | 426.733,32 |

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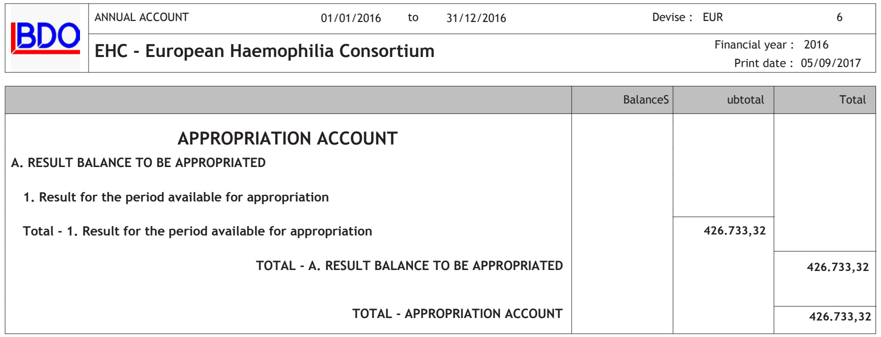

BDO

ANNUAL ACCOUNT 01/01/2016 to 31/12/2016 Devise : EUR 6

**EHC - European Haemophilia Consortium**

Financial year : 2016
Print date : 05/09/2017

| | BalanceS | ubtotal | Total |
|---|---|---|---|
| **APPROPRIATION ACCOUNT** | | | |
| **A. RESULT BALANCE TO BE APPROPRIATED** | | | |
| 1. Result for the period available for appropriation | | | |
| Total - 1. Result for the period available for appropriation | | 426.733,32 | |
| **TOTAL - A. RESULT BALANCE TO BE APPROPRIATED** | | | 426.733,32 |
| **TOTAL - APPROPRIATION ACCOUNT** | | | 426.733,32 |

# SPONSORS

**PLATINUM SPONSORS:**

Bayer, CSL Behring, Novo Nordisk, Pfizer, Roche, Shire, Sobi

**GOLD SPONSORS:**

Biomarin, Grifols

**SILVER SPONSORS:**

Alnylam, Biotest, Kedrion

# Bayer’s passion for patients

## Committed to advancing the treatment of **hemophilia**

Bayer is dedicated to helping patients with hemophilia through the **advancement of recombinant factor VIII (rFVIII) therapeutic options** and expanding the development of innovative technology, including **antibody technology**, **gene therapy**, and **gene editing**.

# An overview of CSL Behring's industry symposium at the European Haemophilia Consortium (EHC) Annual Conference

On 8th October 2016, CSL Behring hosted its first symposium at the European Haemophilia Consortium (EHC) 29th Annual Conference in Stavanger, Norway. Chaired by Professor Paul Giangrande, a panel comprising clinician Professor Pål André Holme, market access consultant Christian Hill, and two European patients, Anders Molander from Sweden and Dr Radosław Kaczmarek from Poland, presented their views on "Advancing the provision of haemophilia care in Europe: reviewing the patient need".

The morning symposium provided a welcomed patient-centric approach and had an impressive attendance of around 110 delegates. The proceedings generated a lively discussion, covering topics including patient views on the importance of trough levels, the advantages of extended half-life (EHL) products versus gene therapy, and how regulators can assess the value of new treatments that offer step-wise innovation.

Professor Holme presented an introduction to the historical background of haemophilia and its treatment, moving forward in time to provide details of current treatments as well as future avenues under investigation. His presentation touched on the evolution of patient management, highlighting that prophylaxis is now considered the optimal standard of care for patients with severe haemophilia, to prevent bleeding and progressive loss of mobility. Professor Holme also emphasised the importance of individualised care and the rationale for adopting this approach.

Each patient panellist presented their personal experience of treatment. Both expressed gratitude and hope at the treatment advancements that have taken place within their lifetime. Anders Molander provided insight into his childhood experience of treatment, reflecting on how lucky he had been to receive excellent physiotherapy, despite his reluctance at the time. Commenting on the current situation in Sweden, he noted that home treatment is now a key element of care. Dr Radosław Kaczmarek acknowledged the achievement of the first generation of haemophilia patients without joint damage. However, he noted that it has been hard to reach the point in Poland where patients are provided with the treatment that is right for them. He stated that barriers still need to be broken in order to move away from the tendency of healthcare officials to standardise care for all patients.

Christian Hill provided an overview of the processes of tendering and Health Technology Assessment to assess the value of treatments referring to a recent paper published by the EHC in which 38 countries were surveyed on their procedures for tenders and procurement. He stressed the importance of early involvement from patient organisations in the tendering and procurement process, if improvement in patient access is to be realised. He outlined potential barriers to the approval of innovative treatments, especially those that offer step-wise innovation, and raised the question of how big a treatment benefit needs to be in order for a new therapy to be considered worthy of approval. He explained the importance currently placed on the different aspects of a treatment during value assessment, and touched on ways this could be improved. He also noted the problems that can occur when it comes to patient access if regulatory submissions are rushed in order to achieve expedited approval.

CSL Behring

# Together we are driving change in haemophilia

Our support for advocacy activities is essential to realising our goal of addressing unmet needs in haemophilia care.

We work with haemophilia communities around the world and in close collaboration with important patient organisations such as the European Haemophilia Consortium (EHC). By supporting projects in social, economic and political environments, we aim to raise awareness together and ultimately improve haemophilia care.

Learn more at **novonordisk.com/changinghaemophilia**

changing haemophilia™

novo nordisk®

# Partnering to Change the World for People with Haemophilia

## Focused support services and tools that deliver real-world innovation to the haemophilia community

At Pfizer, our goal is to provide support for patient-centred initiatives and tools that deliver real-world, practical innovation to support the day-to-day lives of people living with haemophilia.

Through understanding patient lifestyles and how to adapt activity to the individual, we are working to raise awareness of the role of sport, exercise, and active lifestyles in supporting joint health maintenance and patient outcomes.

**Our programmes aim to provide patients and healthcare teams with practical tools and education to support people with haemophilia to lead a full and active life**

MILES FOR HAEMOPHILIA
YOUR PERSONAL BEST!

Haemoassist®2*

Pfizer Ultrasound Programme
Innovation and Imaging in Haemophilia

PHYSIOTHERAPY SCHOOL FOR HAEMOPHILIA

ASPIRE and IGLC Grant Programmes†

To find out more about our activity and active lifestyle support services and tools, contact your local Pfizer team

Job Code: PP-GIP-EUR-1014
Date of preparation: June 2017

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Haemoassist® 2 is a CE-marked medical device in Europe, currently available in Germany, Spain and Austria. Please note that not all Pfizer Programmes may be available in your country. For more information, please contact your local Pfizer representative.

IGLC: Independent Grants for Learning and Change.

†Programmes for HCP use only

Pfizer Haemophilia

These programmes have been initiated and funded by Pfizer

Roche

# *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*

*Let's continue to reimagine haemophilia A, together*

NP/ACE/1708/0028


Jeison
Hemophilia A

# Guided by the haemophilia community in making a pioneering journey together

When Sobi™ re-entered the haemophilia community 4 years ago, we set out on a journey together, with the community as our guide and with a mission to increase access to effective treatment and to make a real difference for people with haemophilia and their families. Building on guidance from the community, we have been able to make what we hope are significant contributions and to have created a solid foundation on our shared journey to continue to improve diagnosis, treatment and care for people with haemophilia. As of 2016:

### First extended half-life treatments available in 13 European countries

Thanks to the guidance of the EHC and its members, we have been able to make the first extended half-life treatment for haemophilia A available in 13 European countries; and for haemophilia B available in 6 countries, in the first year of their authorisation.

Thanks to the guidance of the EHC and its members, we were able to work with the national healthcare systems to make these extended half-life treatments available, on average, almost 6 months earlier than expected across the different countries.

### 14,800 people treated in 40 countries through a donation by Sobi and Bioverativ to the WFH humanitarian aid programme

Thanks to the guidance of the haemophilia community represented by the WFH, we have doubled the number of children treated in the countries where the programme is active today.

Thanks to the work carried out by the WFH, the extended half-life treatments have reached people in a large number of countries where haemophilia diagnosis, treatment and care is still in its early stages.

hellohaemophilia.com

**Now and in the future:** We intend to continue this journey with the community as our guide. The PARTNERS programme, initiated by the EHC, has called upon companies and other stakeholders to work together to secure affordable access to treatment on a sustainable basis in several countries where it is not yet available. Sobi stands behind this initiative to make it possible to elevate treatment standards in countries where healthcare systems are not yet able to treat to the recommended effective minimum.

We intend to keep listening. We want to learn and to be guided by the community. We invite you to continue to tell us what is important, so that we can find solutions and seek improvements in diagnosis, treatment and care together.

Almost every conversation begins with a "hello" – a warm greeting that asks for nothing in return but can lead to endless possibilities. Great things can come from a "hello", from an exchange of ideas and unique points of view, which can contribute to the shared solving of a shared challenge. We invite you to continue to guide us, and we will do our best to continue to listen. www.hellohaemophilia.com

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Date of preparation: June 2017. NP-2472

sobi
Pioneer in Rare Diseases

Pioneering New Research in Haemophilia A

At BioMarin, we develop best-in-class treatments that provide meaningful advancements for people with serious and life-threatening rare genetic diseases. Our 20-year track record of bringing innovative technologies to disease areas has enabled strong, long-standing relationships with the communities we serve.

Today we are focused on new technologies for people living with haemophilia A through the development of our investigational gene therapy. Our development team includes Dr. Barrie Carter, Dr. Gordon Vehar and Dr. Wing Yen Wong, leading scientific and clinical experts who have pioneered our gene therapy program.

GENE THERAPY BY

BIOMARIN®

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Changing the Conversation

inhibitors in hemophilia A

Important information about how inhibitors can impact anyone living with hemophilia A

**Inhibitors are the main treatment challenge in hemophilia A today[1]**

Inhibitors are the most serious complication of hemophilia treatment in which the body's immune system develops antibodies (inhibitors) to the infused factor used to treat bleeding episodes.

**How prevalent are inhibitors?**

ALL patients with hemophilia A are at risk of developing inhibitors, regardless of age and disease severity[2,3]

↓

~35% of people with hemophilia A may develop an inhibitor[4]

↓ ↓

~60% may not be aware they have an inhibitor[2]

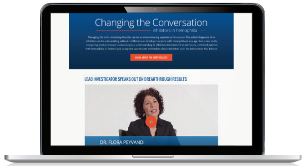
~25% will deal with that inhibitor for life[5]

**Learn more about inhibitors and recent study results**

At InhibitorInfo.com, you can find the latest information about inhibitors, including:

① Summaries of up-to-date inhibitor data and study results

② Videos of leading hematologists discussing actual topics of interest



Visit InhibitorInfo.com

**1.** World Federation of Hemophilia. What are inhibitors?. Published 2009. Accessed May 10, 2016. **2.** Soucie JM, Miller CH, Kelly FM, et al. A study of prospective surveillance for inhibitors among persons with haemophilia in the United States. Haemophilia. 2014;20(2):230-237. **3.** Hay CRM, Palmer B, Chalmers E, et al. Incidence of factor VIII inhibitors throughout life in severe hemophilia A in the United Kingdom. Blood. 2011;117(23):6367-6370. **4.** Peyvandi F, Mannucci PM, Garagiola I, et al.A randomized trial of factor VIII and neutralizing antibodies in hemophilia A. N Engl J Med. 2016;374(21):2054-2064. **5.** Valentino LA, Kempton CL, Kruse-Jarres R, Mathew P, Meeks SL, Reiss UM. US guidelines for immune tolerance induction in patients with haemophilia A and inhibitors. Haemophilia. 2015:1-9. doi: 10.1111/hae.12730.

GRIFOLS

EHC

european haemophilia consortium

www.ehc.eu