



| EHC NEWSLETTER

APRIL 2015

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EHC Newsletter April 2015

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President and CEO Report

World Haemophilia Day 2015 – a double celebration!

For World Haemophilia Day (WHD) this year, the European Haemophilia Consortium (EHC) and its



Mr Brian O'Mahony is the President of the EHC



Mrs Amanda Bok is the EHC CEO

community had two reasons to celebrate.

First, following on exactly one year after we highlighted the important new haemophilia recommendations from the Wildbad Kreuth III initiative that were published in [*Haemophilia in spring 2014*](#), these were taken up by the Ministers of the Council of Europe (CoE) and elaborated into an official resolution, which was adopted in time for WHD 2015. The resolution recommends that governments of the

State Parties to the Convention on the Elaboration of a European Pharmacopoeia¹ (37 out of the 47 CoE Member States) ‘take appropriate measures to step up the promotion’ of [*six out of the seven original Wildbad Kreuth III principles*](#). This is an historic achievement and the first Council of Europe Resolution directly on haemophilia and rare bleeding disorders in 35 years! It adds significant political weight to these recommendations and is an important aid to National Member Organisations (NMOs) working with their governments to improve treatment and care nationally.

Second, the EHC unveiled the findings of its 2014 survey of 38 European countries on tenders and procurement at its WHD event held in Dublin, Ireland, on 16 April. The most important of these findings shows that when clinicians and patients are both formally involved in the procurement process, the outcome for the overall health system improves in a statistically significant way. The survey has been accepted for publication by *Haemophilia* and will be available soon. We would like to sincerely thank the 38 countries who responded to this important survey. [*More information about the WHD event is available here*](#). This year the EHC will conduct a survey on haemophilia care in Europe, which is an area the EHC examines every three years.

MSM Position Statement

Last month the EHC issued a position statement on blood donation and MSM (men who have sex with men) in response to an increasing number of European governments reviewing their national policies. [*The EHC position*](#) is that this is *primarily* a risk management issue – not an issue of social policy or politics or equality – which should be dealt with nationally in the same way as any other existing or proposed donor deferral measure for blood or plasma donors, namely: based on data and scientific evidence, and driven by the expert opinion of blood transfusion services

¹ Austria, Belgium, Bosnia and Herzegovina, Bulgaria, Croatia, Cyprus, Czech Republic, Denmark, Estonia, Finland, France, Germany, Greece, Hungary, Iceland, Ireland, Italy, Latvia, Lithuania, Luxembourg, Malta, Montenegro, Netherlands, Norway, Poland, Portugal, Romania, Serbia, Slovak Republic, Slovenia, Spain, Sweden, Switzerland, “the former Yugoslav Republic of Macedonia”, Turkey, Ukraine and United Kingdom.

Round Table on inhibitor risk in haemophilia A

On 2 March the EHC held its 24th Round Table on ‘Product related risk for inhibitor formation in haemophilia A’ in Brussels. Attended by more than 50 participants, the Round Table focused on current scientific findings on factor-related risks for inhibitor development in previously untreated patients (PUPs) with haemophilia A. The results from various studies including SIPPET², RODIN³, FranceCoag⁴, UKHCD⁵ and EUHASS⁶ were presented and discussions included a critical appraisal of the available data, an epidemiologist’s view of the risk of inhibitors and how they should be assessed in the future, and a call for patients to actively report adverse events when they occur. For more information please see pg 11 or visit the [EHC website](#).

First meeting of the informal ‘MEP Group of Friends of Haemophilia and Rare Bleeding Disorders’

On 24 March the newly formed ‘MEP Group of Friends of Haemophilia and Rare Bleeding Disorders’ held its first meeting in the European Parliament in Brussels. Chaired by long-time EHC supporter Nessa Childers MEP (Ireland), the meeting focused on setting the group’s 2015 agenda, discussing upcoming potential legislative reforms and reviewing current and future Round Tables. Current members of this group include Dr Cristian-Silviu Bușoi (Romania), Dr Miroslav Mikolášik (Slovakia) and Dr Paul Rübig MEP (Austria).

European Reference Networks

The European Commission Expert Group on Rare Diseases (CERD) continues its efforts to set the operational framework for Rare Disease European Reference Networks (RD ERNs).

Work has already begun on outlining the standards and guidelines for ERNs. These will be contained in the ‘Assessment Manual and Technical Toolkit⁷’, which independent assessment bodies will use when reviewing RD ERN applications.

CERD, at its last meeting in March, also reviewed a proposed Addendum, which saw a patient-centred approach to RD ERNs and the grouping of rare diseases into 22 thematic networks, including ‘rare haematological diseases.’ A revised version of this Addendum will be proposed for adoption at the next meeting in June. The EHC position was that 22 thematic networks would not be feasible, in particular not for our disease area. However there was no room to expand this number and the unspoken concession seems to be that ‘informal sub-networks’ may be possible within a larger thematic network.

² Survey of Inhibitors in Plasma-Product Exposed Toddlers.

³ Research of Determinants of Inhibitor Development among PUPs with haemophilia.

⁴ FranceCoag Network is a cohort of French patients suffering from inherited deficiencies of coagulation proteins, such as haemophilia A and B, Von Willebrand’s disease or other rare inherited bleeding disorders.

⁵ The United Kingdom Haemophilia Centre Doctors’ Organisation.

⁶ EUHASS is a pharmacovigilance program to monitor the safety of treatments for people with inherited bleeding disorders in Europe.

⁷ The European Commission awarded the tender for the development of that toolkit to a consortium made up of the European Organisation for Rare Diseases (EURORDIS), the European Hospital and Healthcare Federation (HOPE) and Accreditation Europe; this consortium is called Partnership for Assessment of Clinical Excellence in European Reference Networks (PACE-ERN).

The European Commission expects to issue a call for ERNs in December 2015, assessment would take place from March-May 2016 and the first approvals would take place around July 2016. The next conference on RD ERNs will take place on 8-9 October in Lisbon, Portugal; it is meant to feature a plenary on the 8th and hands-on workshops for ‘mature networks’ on the 9th.

More information about RD ERNs and the list of national members of the Board of Member States of the ERNs will be sent to all NMOs in the next EHC Quarterly Policy Report.

European Commission reviews communication on similarity and significant benefit for orphan drugs

The European Commission has announced that it will be revising its ‘Communication on Regulation (EC) No 141/2000 of the European Parliament and of the Council on orphan medicinal products (2003/C 178/02),’ which outlines the criteria for designation, authorisation and marketing exclusivity. In particular, the revision will focus on the important definitions of similarity and significant benefit. Having advocated with the European Commission and the European Medicines Agency on this issue for the past two-three years, we very much welcomed this revision process and will use the opportunity of submitting a written position in advance of the public consultation process, which will take place during the second half of 2015. The adoption of the revised Communication is expected in the first quarter of 2016.

NEW: Leadership Conference 2015

The EHC will hold its first Leadership Conference pilot from 30 September to 2 October in Belgrade, Serbia, to be immediately followed by the EHC Annual Conference from 2-4 October in the same venue. The programme will focus on the three main themes of governance and succession planning; NMO financing and fundraising; and tenders and procurement. We have invited every NMO to send their senior leader as well as a youth leader and a staff person and we very much look forward to the multi-faceted exchange of experiences and best practices in these areas. We are grateful to Baxter, Pfizer and Sobi for supporting this initiative. For more information about the EHC leadership conference go to pg 22.

NEW: European Inhibitor Network 2015-2017

We are pleased to inform you that the EHC is launching a multi-annual programme focused on people with haemophilia who have inhibitors. This group within our community has been historically under-served and has significant unmet needs. It is time to change that! For this reason we have created a multi-faceted programme called the ‘European Inhibitor Network,’ which seeks to better understand and support the needs of patients with inhibitors as well as their families and care-givers. To begin the work of this programme, the EHC will conduct a series of needs assessments within its community as well as hire a full-time professional staff resource to drive and implement our work in this area. If you are interested in hearing more about this programme or sharing your views, opinions or ideas, we would love to hear from you! More details about this programme will be shared soon and will also be presented during the first parallel workshop of the EHC Annual Conference in Belgrade, Serbia, later this October. This programme is made possible by an educational grant from Baxter.

NEW: Introducing Aislin Ryan, EAHAD-EHC Project Consultant

We are delighted to welcome Ms Aislin Ryan into the small and dynamic EHC team as well as into her unique position as EAHAD-EHC Project Consultant, a joint position between the EHC and the European Association for Haemophilia and Allied Disorders (EAHAD) to support and further the collaboration between our two organisations. The EHC interviewed Aislin for this newsletter and we invite you to learn more about her on pg 13. Please join us in giving Aislin a warm welcome!

EHC News

Comprehensive care for bleeding disorders: An overview of comprehensive care services and professionals

By Laura Savini, EHC Communication and Public Policy Officer

In 2015, the European Haemophilia Consortium Newsletter will look at different healthcare professions involved in the comprehensive care of haemophilia and other bleeding disorders. For this first edition we are looking at dental care and nursing. Laura Savini talked to Dr Alison Dougall from the Dublin University Dental Hospital on dental care in haemophilia and to Ms Marlies Schrijvers and Ms Nanda Uitslager on nursing in haemophilia comprehensive care.

Dental Care

Alison Dougall, a Consultant in Special Care Dentistry at the Dublin University Dental Hospital is no stranger to dental care in haemophilia. She has been treating people with bleeding disorders for many years and is now a candidate for a PhD on 'The value of dental care for people with haemophilia.'



Alison Dougall is a Consultant in Special Care Dentistry at the Dublin University Dental Hospital (photo courtesy of Alison Dougall)

"The mouth is an integral part of the body and is essential to eat, speak, kiss and carry out a normal and fulfilling life. Individuals affected by mouth diseases and bad mouth hygiene often suffer not only physically but also emotionally, as they can have associated low self-esteem, which will impact their social, work and family life," states Alison. This is why, in her opinion, mouth hygiene and dental care are so important and why it is very unfortunate that these issues are often

overlooked when treating people affected by bleeding disorders.

Like haemophilia care, general dental care has much evolved in the past 25 years. While the old-fashioned approach to dental care was to treat the problem whenever it presented itself (for example, extract a decayed tooth), modern dental care views the need to fix any such problem as a failure. In fact, just like haemophilia prophylaxis for joint care, modern dental care focuses on prevention and minimalistic interventions (such as protective sealants and varnishes and in-depth cleaning) that if performed on a regular basis can avoid the need for costly and more invasive interventions altogether. Alison points out that these days basic dental care is

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relatively inexpensive and that the only really expensive procedures are tooth extractions, gum surgery, bridges and implants or cosmetic work. So, the objective of any dentist will be to prevent the need for those issues in patients. This is also a mission of the World Health Organisation, which has designated tooth decay and gum disease as an urgent problem because they not only carry risks like the development of infection and impact on general health, but also have a pretty significant impact on the healthcare system budget.

Another development, which has a considerable impact for people with bleeding disorders seeking dental care, is that nowadays all dental students are taught how to treat patients with a higher bleeding profile (this includes not just people with bleeding disorders, but also people who take particular medication such as anti-coagulants). This means that any qualified dentist can treat a person with a bleeding disorder. However, Alison stresses, it is key that the dentist receives information beforehand about the patient so that he or she can be treated properly in partnership with the haemophilia team.

So, why is it trickier for people with bleeding disorders to receive adequate dental care?

According to Alison, people with bleeding disorders have an innate fear of dentists, just like the rest of the population, but with one big difference. Each person with a bleeding disorder knows a *horror story* about a dental procedure going terribly wrong in the past. Unfortunately, these episodes still do take place around the world. For instance, Alison told the story of a person with haemophilia who died during a tooth extraction in Latin America just last year. It is then understandable why fear is perhaps the biggest barrier to proper dental care for people with bleeding disorders.

Nevertheless she stresses that when dentists use well-documented updated haemophilia protocols, dental techniques and products alongside factor cover when necessary, even the most complex dental surgeries can be performed safely without risk.

Alison also insists on debunking an all-too-recurring haemophilia myth, which is that people with bleeding disorders will bleed from the gums as part of their condition. This is not correct, in fact people with haemophilia who bleed from the gums do so because they are most likely affected by some type of gum disease due to poor dental hygiene.

Alison also insists on debunking an all-too-recurring haemophilia myth, which is that people with bleeding disorders will bleed from the gums as part of their condition. This is not correct, in fact people with haemophilia who bleed from the gums do so because they are most likely affected by some type of gum disease due to poor dental hygiene. In fact, Alison deplores the fact that people with bleeding disorders are often given incorrect advice on how to care for their teeth - ranging from not brushing their teeth when they bleed to brushing their teeth with a sponge. These practices will only increase the long-term chances of tooth and mouth diseases, which in return will only increase the likelihood of expensive and invasive dental treatments. Furthermore, she stresses that with the advent of specialised toothpastes, gels, mouthwashes and medicated versions of these products, people with bleeding disorders have the option to treat early symptoms of a mouth disease in a really non-invasive way. She also notes that people with bleeding disorders will sometimes choose to treat dental problems with factor alone or suffer long periods of toothache before seeking care. Again, in her opinion, this is not the appropriate response. People with gum or mouth bleeds and associated pain need to seek the opinion of a dental professional early and take adequate pain medication whilst arranging the necessary dental care.

In Alison's opinion, people in general want a few things: the freedom to choose their dentist and the peace of mind to know that their dentist visit will go well and they will be treated appropriately.

have experienced. Furthermore, although the risk of bleeding following routine or simple non-surgical care is very low, the fear of bleeding after dental procedures is high because they do not have home treatment like people with a severe bleeding disorder. This is why people with mild bleeding disorders will most likely avoid seeking any dental care at all until the problem develops into something more significant, which in turn will only make the experience more traumatic.

Finally, Alison notes that somewhere along the way dental care split from other medical specialties and is now viewed by other healthcare professionals as a separate area of medicine. Alison noted that often medical teams in comprehensive care centres do not have as much knowledge about dental care as they have in other medical areas related to bleeding disorders. This situation can have a big impact on patients because they run the risk of not automatically receiving information about the risk profile of dental procedures. It then becomes more difficult for them to decide which procedure to choose. This is why it is very important to take a holistic approach to the care of bleeding disorders. She notes that for the reasons mentioned above, patients with bleeding disorders do often need some help and encouragement to take care of their dental health and this can be made easier if patients know that different specialists work together to ensure they receive the most appropriate care. In Alison's opinion, having your dental health in check really helps to have peace of mind.

How can dentists be better integrated into comprehensive care?

In terms of dental care offered by comprehensive care centres in Europe, Alison referred to the EHC survey of 35 countries carried out in 2012, which showed that the landscape for dental care in Europe is very diverse. From her own experience, centres can have very different attitudes towards dental care with some centres expecting their patients to solely seek dental care in the comprehensive care centre, while others offer only emergency dental care and their patients should see a regular dentist for

regular visits. Finally in some hospitals *non-specialist* dentists provide advice on how to conduct dental care for people with bleeding disorders. In Alison's opinion, people in general want a few things: the freedom to choose their dentist and the peace of mind to know that their dentist visit will go well and they will be treated appropriately. For this purpose, Alison believes that it is really important for comprehensive care centres not only to provide specialised dental services for emergencies but also to provide information to dentists about the bleeding profile of their patients and to inform patients on the risk profile of different dental procedures so that patients can make informed decisions. In her opinion, improvements can come from doctors, nurses and social workers having a better understanding of dental care and the risk profile of various procedures. In addition patients' organisations have a role to play by developing information resources to debunk myths and provide adequate information to their members.

Fear also seems to be an important barrier to dental care for people with mild bleeding disorders, a category of patients that is unfortunately often overlooked in this regard. This is due to several factors, the first being that an important number of people affected by mild bleeding disorders were diagnosed following a surgical dental procedure that involved an important amount of bleeding. Often this is one of the few bleeding episodes that this group of patients will



Alison (right) attended the EHC New Technology Workshop in November 2014

Alison has had a great experience working with the Irish Haemophilia Society (IHS). The collaboration has been ongoing for years and together they have developed many tools to improve education about dental care. For instance, they developed leaflets and fact sheets that patients can take to their dentists for guidance. They have also organised advocacy activities to access increased funds so that people with lower incomes can receive government-sponsored dental care. Furthermore, whenever the IHS organises an event, it involves dentists and hygienists to provide information about dental care and to provide training to adults and children alike on good dental hygiene practices. For example, Alison attended women's workshops to discuss the topic. She notes that dental care is a particularly important issue for women who have grown up with either a father or brother having a bleeding disorder and saw the effects of poor dental care. Other women affected by bleeding disorders ask a lot of questions about their own worries about bleeding and also in regards to cosmetic dental procedures, such as the safety of cosmetic teeth whitening and appropriate dental care and orthodontic braces for their children.

What are the biggest challenges in dental care for haemophilia today?

For Alison, one of the most pressing challenges for dentists taking care of haemophilia patients is to demystify dental care and debunk myths. This can be achieved through collaboration amongst healthcare professionals and with patients' organisations. Another challenge is to take care of older patients. *"We are currently seeing the first generation of patients with bleeding disorders growing old with their own teeth. As they get older, these patients are also affected by other conditions that come with old age and are more likely to take medication that has an impact on their mouth. It is important for these patients to get their oral health regularly assessed and taken care of," states Alison.* Finally, it is extremely important to ensure that people with bleeding disorders understand that dental care can be safely managed and that preventive measures are easy and cost-effective.

In terms of collaboration, the World Federation of Hemophilia has a dental working group and a multidisciplinary working group that includes dental care. However, Alison hopes that a European-wide collaboration can be established between healthcare professionals treating haemophilia and dentists to increase the holistic approach to haemophilia care.

Nursing Care

For most people, including people with a bleeding disorder, the nurse is the first point of contact when visiting a hospital and when seeking medical advice. The nurse is the link between the various healthcare professionals and the patient because they also have a good understanding of how hospital services are structured and who is charge of what. Nurses are also often seen as being more approachable than doctors. Thanks to this, nurses will usually play a key role in bridging any potential gaps in understanding between healthcare professionals and patients. This is why nursing professionals are at the centre of comprehensive care in bleeding disorders.



Marlies Schrijvers is a PhD student in Nursing Science (photo courtesy of Marlies Schrijvers)

This is how Ms Marlies Schrijvers and Ms Nanda Uitslager, both working at the Van Creveldkliniek in Utrecht, describe the role of the *haemophilia nurse*. Marlies is a PhD student in nursing science. She is the chair of the European Nurses Committee at the European Association of Haemophilia and Allied Disorders (EAHAD) and a

Haemophilia nurses are not only the first point of contact for patients but they are often more attuned to patients' needs and to their general state of wellbeing.

member of the committee for the Certification of European Haemophilia Treatment Centres with EUHANET. Nanda is an advanced nurse practitioner at the Van Creveldkliniek. She is also a member of the organising committee of the annual Bayer HealthCare Hemophilia Nurse Conference and the bi-annual Van Creveld symposium.

What is the role of the haemophilia nurse?

Both Marlies and Nanda explained that haemophilia nurses are not only the first point of contact for patients but they are often more attuned to patients' needs and to their general state of wellbeing. For example, patients can easily access the haemophilia nurse if something is wrong and receive the support they need.

The nurse, just like other professionals involved in comprehensive care, sometimes follows patients throughout their entire life span. Both Nanda and Marlies recognised that each patient has different needs depending on their life phase. For example, in children, education of the parents is one of the most critical issues to avoid needle phobia. Once these children become adolescents, nurses can start negotiating with the patients directly and develop a relationship with them. In older patients, it is very important to be respectful of their knowledge and experience, but also to bring that knowledge up to date.

What are the main challenges in haemophilia care from a nursing perspective?

Despite current effective therapy, Marlies and Nanda also see new challenges in nursing care. Usually people with a more severe form of bleeding disorders have up-to-date medical records. Nowadays, due to high healthcare costs and co-payments, people with mild and moderate haemophilia skip their regular check-ups. This could be a problem when an emergency occurs, because their health information will not be current. This fact will make it more difficult for healthcare professionals to treat them, potentially leading to unnecessary medical complications. This problem is currently intensified in countries like the Netherlands due to the financial crisis as patients are required to make a co-payment for healthcare services that they access. Nurses can remind patients of the importance of getting regular check-ups. Another issue noted by both Nanda and Marlies is the problem of increasing weight and obesity amongst people with bleeding disorders, which has a negative impact on joints and requires higher levels of prophylaxis.

The objective of the haemophilia nurse is to help the patient to best manage his or her condition and live an autonomous life and to remind them to maintain regular contact with the treatment centre.

The objective of the haemophilia nurse is to help the patient to best manage his or her condition and live an autonomous life and to remind them to maintain regular contact with the treatment centre.



Nanda Uitslager is an advanced nurse practitioner at the Van Creveldkliniek (photo courtesy of Nanda Uitslager)

Finally, with the advent of better treatment regimens and the arrival of longer-acting products on the market, nurses think that children and adolescents will find it more difficult to identify a bleed. This could result in problems with adherence to prophylaxis. Nurses need to work closely with physiotherapists to teach patients how to recognise a bleed and what procedure to follow.

What are the differences in haemophilia care across Europe?

With regard to the European landscape, while working with EAHAD and EUHANET on the certification of comprehensive care centres, Marlies discovered that the way nursing services are organised differs quite a bit depending on the country and on the centre. Besides differences in the staff capacity of each centre, some countries grant nurses more responsibilities. This means that in Europe the activities that nurses are allowed to perform vary. For example, in Germany only doctors can administer prophylaxis, while in the United Kingdom nurses can also do this. Currently, there is no standard training for haemophilia nurses in Europe. The UK offers some specialised courses, yet most nurses learn the specialisation on the job and by attending conferences. European collaboration is a current objective of the EAHAD Nurses Committee. Besides working together for the past two years, the EAHAD conference has featured multidisciplinary sessions where doctors, nurses and other healthcare professionals speak about and discuss key aspects of the treatment of bleeding disorders from each of their professional perspectives. As there is a lot of variety of practices in Europe, the EAHAD Nurses Committee is working on a curriculum and guidelines for haemophilia nurses. The objective of this project is to provide patients across Europe with the same high quality care.

With regard to their relationship with patients, both Marlies and Nanda have regular contact with patients in their daily work and they will be in touch with the patient society.

Finally, Marlies and Nanda concluded by stating that being a haemophilia nurse is a rewarding profession that allows them to build a long-lasting relationship with patients. For them it is important to develop the role of the haemophilia nurses so that all people with bleeding disorders in Europe all can have access to high quality haemophilia nursing care.

EHC holds Round Table on factor concentrate-related inhibitor formation in haemophilia A

By Dan Farthing-Sykes, CEO Haemophilia Scotland

The European Haemophilia Consortium (EHC) have organised another one of their Round Table meetings. These events give patient representatives an invaluable chance to hear from some of the leading clinicians on a key issue facing people with bleeding disorders in Europe.

The March 2015 meeting tackled the issue of inhibitors in haemophilia A. If your immune system responds to a clotting factor product and stops it from working, it is called an inhibitor. It is currently estimated that an average 30 per cent of people who regularly take clotting factor products will develop an inhibitor.

A lot of the discussion focused on whether different types of products or treatment regimens had any impact on the risk of developing an inhibitor – especially when someone has not had treatment before. Previously Untreated Patients are often abbreviated to PUPs in these discussions.

Recombinant or Plasma Derived Products

We heard that the largest retrospective study, the Research Of Determinants of Inhibitor Development among PUPs with haemophilia (RODIN) does not show conclusively whether there is a difference in inhibitor risk between plasma derived and recombinant products.

Therefore, a randomised clinical trial, known as the Survey of Inhibitors in Plasma-Product Exposed Toddlers (SIPPET study), has been started. The SIPPET study has looked at an effective sample of 260



Dan Farthing-Sykes is the CEO of Haemophilia Scotland (photo courtesy of Haemophilia Scotland)

people with haemophilia from all over the world. To date, 29 per cent of people enrolled in the study developed an inhibitor. The study is testing the idea that plasma-derived products might be half as likely to cause an inhibitor. However, the study is still ongoing and has not produced an answer yet.

Second or Third Generation Products

The RODIN Study indicated that there might be an increased inhibitor risk between second generation products and third generation products. Studies have been conducted in France⁸, the UK⁹, and Canada¹⁰ to try and confirm the finding. The combined studies showed a statistically significant increased risk for PUPs who were on second generation products. However, this data needs further checking for potential bias so the results are being treated with a lot of caution. At the moment there is not a clear explanation for why there might be an increased risk. One of these studies also suggested an increased risk associated with ReFacto AF but the low numbers of people on the product means that these figures are not significant and need further investigation.

European Monitoring

The European Haemophilia Safety Surveillance (EUHASS)¹¹ project looks at the question of inhibitors in a different way. It is a Europe-wide adverse events surveillance system. The participating haemophilia centres regularly report on the number of inhibitors and the number of patients reaching their 50th treatment (this is because it is noted that patients develop inhibitors more frequently in their first 50 exposures to the treatment). They found a slightly lower rate of inhibitors at about 26 per cent.



Prof Pier-Mannuccio Mannucci presented the SIPPEL Study during the Round Table

There was a suggestion that it would take a randomised controlled trial (RCT), which randomly assigns products to previously untreated patients, to give stronger results. However, concerns were raised about whether this would be possible while still giving patients fully informed choice.

Patients and Patient Organisations

Thomas Sannié, from the Association Française des Hémophiles (AFH), argued that patients play a vital role in surveillance after marketing authorisation of any product. This requires engaged and well-informed patients who are prepared to make reports. He described the work of the AFH on a project that developed tools to help make reporting adverse events, including inhibitors, part of the

⁸ Calvez, Chambost, Claeysens-Donadel, et al. Recombinant factor VIII products and inhibitor development in previously untreated boys with severe haemophilia A. *Blood* 2014; 124(23):3398-3408.

⁹ Collins, Palmer, Chalmers, et al. Factor VIII brand and the incidence of factor VIII inhibitors in previously untreated UK children with severe haemophilia A, 2000-2011. *Blood* 2014; 124(23):3389-3397.

¹⁰ Vézina, Carcao, Infante-Rivard et al. Incidence and risk factors for inhibitor development in previously untreated severe haemophilia A patients born between 2005 and 2010. *Haemophilia* 2014; 20:771-776.

¹¹ EUHASS is a pharmacovigilance program to monitor the safety of treatments for people with inherited bleeding disorders in Europe.

culture of the bleeding disorders community in France. This includes a new website, which guides patients through the process.

Conclusions

In light of all the presentations and discussions, there was a general consensus that additional evidence is needed to determine whether any single product increases the risks of inhibitor development. Although some speakers claimed that when in doubt physicians should abstain from prescribing a particular product, if alternatives are available, others insisted that the only way to be certain whether a particular product increases the risk of inhibitor development is to run a RCT.

You can find the full report of the event on the [EHC website](#).

Meet Aislin Ryan, EAHAD – EHC Project Consultant

Aislin Ryan interviewed by Laura Savini***

In early February Aislin Ryan joined the European Association for Haemophilia and Allied Disorders (EAHAD) and the European Haemophilia Consortium (EHC) as a Project Consultant. Aislin talks more about her background and current work.



*Aislin is the EAHAD-EHC Project Consultant
(photo courtesy of Aislin Ryan)*

Aislin is a native of Canada and while new to Europe, she is not new to the bleeding disorders community having worked previously for the World Federation of Hemophilia (WFH) in her hometown, Montreal. Aislin spent five years at the WFH, first in the Communications Department and then in the Programs Department where she managed the International Haemophilia Training Centre (IHTC) Program and the International External Quality Assurance Scheme (IEQAS). In addition to this, Aislin also worked alongside the Program Manager for Europe to support the national member organisations (NMO) in Belarus and Moldova. This is where she gained first-hand experience working with both patients and healthcare providers in Europe and where she was directly confronted with some of the more difficult realities experienced by people with bleeding disorders. At the same time, she was also inspired by the commitment and dedication of the haemophilia

community to work together to improve bleeding disorders care.

In 2012, Aislin left the WFH to pursue a Master's in Public Health at Simon Fraser University in Vancouver, Canada. During her studies, she focused on how to improve the transfer of health information between patients, researchers and policymakers. As part of her degree, she spent three months in Argentina working on a project that used photography as tool for people living with Chagas disease to communicate their experiences to healthcare authorities and researchers. This is particularly complementary to her current position where one of her main projects is based on fostering increased collaboration between patients and healthcare professionals.

For Aislin, working for EAHAD and EHC is the perfect opportunity to combine the theoretical learning from her public health studies with her previous practical experience in haemophilia. A big part of her mandate is to work on joint projects between the EHC and EAHAD to continue to build on the collaboration between the two organisations both on advocacy issues and on the advancement of haemophilia treatment. One initiative she is especially enthusiastic about is a joint project between the EHC, EAHAD and the WFH aimed at improving haemophilia treatment and care in European

countries in need, particularly in Eastern Europe. This is the pilot year for the project with a visit to Estonia and Latvia planned for the fall. *"There is so much experience in each of the three organisations that it seems only natural to combine them in order to achieve the maximum impact. It is clear how important comprehensive care is for haemophilia so by the same token, on an organisational level, collaborating on this kind of project is an excellent way to share knowledge and assist our members' countries in a collective way."*

Aislin is the first staff person at EAHAD and one of her main roles is also to represent the organisation within the bleeding disorders community and increase EAHAD's visibility among healthcare providers working in haemophilia across Europe, including not only haematologists but also nurses, physiotherapists and other medical professionals. This includes updating EAHAD's website, newsletter and other communications materials. Furthermore, Aislin provides support to the EAHAD Executive Committee and Working Parties on their day-to-day work.

"EAHAD's members are active on so many levels, that it is good to have a central point of reference to get alignment on all the projects in which EAHAD takes part. This year will definitely have its challenges, as there is a lot to learn about the particular landscape of haemophilia care in Europe. At the same time it is exciting to be able to part of the development of EAHAD as it continues to grow and broaden its mandate."

"EAHAD's members are active on so many levels, that it is good to have a central point of reference to get alignment on all the projects in which EAHAD takes part. This year will definitely have its challenges, as there is a lot to learn about the particular landscape of haemophilia care in Europe. At the same time it is exciting to be able to part of the development of EAHAD as it continues to grow and broaden its mandate," she states. *"I am also excited to work with the dynamic team at the EHC and be able to contribute to projects targeted both to healthcare professionals and patients. Finally it is a real pleasure to return to working in the haemophilia community, which has always been so warm and welcoming."*

Aislin is based at the EHC office in Brussels and can be reached at aislin.ryan@eahad.org and at +32 (0)2 893 24 89. If you would like more information about EAHAD activities or membership, please do not hesitate to contact Aislin or visit EAHAD's website at www.eahad.org.

*Aislin Ryan is the EAHAD-EHC Project Consultant

**Laura Savini is the EHC Communications and Public Policy Officer

EHC revamps its website

By Laura Savini, EHC Communication and Public Policy Officer

The European Haemophilia Consortium (EHC) was thrilled to unveil our new website this December during the cocktail reception organised to mark our 25th anniversary. The website went live in the first weeks of January. Here we give a short overview of the new design to help orient you to the layout and features.



Home Page

The Home Page is the first point of contact for visitors to the EHC's activities and news. On the top level, just below the menu bar, there is a **Slider** (1) displaying information about EHC activities. This could be the announcement of an event or publication or could highlight a news story.

On the left-hand of the page, just below the sliders, the visitor will find a series of **Posts** (2). These

The screenshot shows the EHC website's main content area with several sections:

- POSTS** (2): A grid of news items. One item is titled "THE PENROSE INQUIRY" with a link to "Report of the Penrose Enquiry gets published". Another item is titled "EHC is looking for a new colleague" with a link to "Job Program". A third item is titled "EHC hosts Round Table on factor concentrate-related inhibitor risk in haemophilia A" with a link to "Read more". A small white box containing the number "2" is located to the left of the first post.
- UPCOMING EVENTS** (3): A list of events with dates and descriptions. It includes "World Haemophilia Day 2015 Event" on April 16, "Round Table on Tenders and Procurement" on June 15, and "Youth Workshop 2015" on July 03. A small white box containing the number "3" is located to the right of the events section.
- IN THE MEDIA** (4): A section showing news articles from external sources. One article is from "Haemophilia News" about the Penrose Inquiry, and another is from "bbc.com" about the Penrose blood contamination inquiry report. A small white box containing the number "4" is located to the left of the media section.
- EHC FACEBOOK FEED** (5): A section showing a post from the "European Haemophilia Consortium" Facebook page. The post discusses the Penrose report and has 3 days ago. A small white box containing the number "5" is located to the right of the Facebook feed section.

are the latest updates both from the EHC and other organisations and can focus on current events. All of these posts are archived in the News section of the website. To the right of this is the **Upcoming Events** section giving an overview of the next three EHC events (3). Below the events, you will find the latest Facebook posts from the **EHC Facebook page** (5). On the left hand-side under **In the Media**, the visitor will be able to consult the latest posts from Haemophilia News (4). This is a Facebook account run by an EHC volunteer that gathers information in English-speaking media relevant to the bleeding disorders community.

Shaping the future for people with congenital bleeding disorders

WHERE TO FIND US

6

OUR MISSION

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

WEBSITE REVISION SPONSOR

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EHC OFFICE

Address: rue de l'Industrie/
Nijverheidsstraat 10, BE-1000, Brussels

Phone: tel.: +32 (0)2 893 24 70
E-mail: office[at]ehc.eu
Website: www.ehc.eu

Finally at the bottom of the page, the visitor will get all the practical details regarding the EHC such as our mission statement, contact details and a map of our office location (6).

Menu Bar

The menu bar is located at the top of the page and remains the same throughout the website. The sections currently visited are highlighted in red.

[HOME](#) [ABOUT EHC](#) [BLEEDING DISORDERS](#) [AREAS OF WORK](#) [EVENTS](#) [NEWS](#) [LIBRARY](#) [CONTACT](#)

About EHC

This is the section of the website where visitors can find all information related to the organisation of the EHC such as its **Mission and Objectives**, its **Membership**, and its **Partners**.

The screenshot shows the EHC website's navigation bar with links to Home, About EHC, Bleeding Disorders, Areas of Work, Events, News, Library, and Contact. The 'ABOUT EHC' menu is expanded, showing sub-links: Mission And Objectives, Membership, Governance, Partners, and Finance And Transparency. A large image of a person in a red shirt is visible in the background.

should be noted that this section is not meant to provide detailed scientific and medical information, but rather is designed to give the general public a better understanding of what bleeding disorders are and what the impact of living with a bleeding disorder is. In each part of the bleeding disorders sections you can find links to additional information on other websites.

The screenshot shows the 'BLEEDING DISORDERS' section of the website. The left sidebar lists categories: About Bleeding Disorders, Haemophilia, Von Willebrand Disease, Rare Bleeding Disorders, Platelet Disorders, Inhibitors, Comprehensive Care, Treatment Options, and Useful Information. The main content area features a large image of a woman with dark hair. A sidebar on the right lists menu items: About Bleeding Disorders, Haemophilia, and von Willebrand Disease.

Areas of Work

The screenshot shows the 'AREAS OF WORK' section. The left sidebar lists three categories: Support And Empower National Member Organisations, Promote Access To Treatment And Care, and Policy-making, Advocacy And Awareness. The main content area contains text about the work carried out by the EHC.

This section, structured according to the EHC strategic objectives, will give the visitor more information about the work carried out by the EHC.

Events

This is where all the information related to EHC Events can be found, be it Round Tables, Workshops, Conferences or World Haemophilia Day events. The Event section contains programs and other logistical information related to EHC events. Any material related to EHC events can be found in the Library Section.

There is also a section related to **Governance**, which provides details about the management of the EHC. Another section gives the visitor more information about the **Finances and the Transparency** of the EHC.

Bleeding Disorders

This is where the visitor can find information about bleeding disorders. It

HOME ABOUT EHC BLEEDING DISORDERS AREAS OF WORK EVENTS NEWS LIBRARY CONTACT Conferences Workshops World Haemophilia Day

UPCOMING



Round Table on Mild Haemophilia

On 30 November 2015, the European Haemophilia Consortium (EHC) will hold its 24th Round Table on 'Mild Haemophilia'. The event will bring together experts from across Europe to discuss the latest research and clinical practice in the field. The event will also provide a platform for participants to share their experiences and insights.

READ MORE **Nov 30**

Conferences table of the year on: "Mild Haemophilia" to all NMOs and selected participants. The event will bring together experts from across Europe to discuss the latest research and clinical practice in the field. The event will also provide a platform for participants to share their experiences and insights.

Workshops

World Haemophilia Day

Round Table on Factor Concentrate-Related Inhibitor Risk in Haemophilia A

The European Haemophilia Consortium (EHC) will hold its 24th Round Table on 'Factor concentrate-related inhibitor risk in haemophilia A' on Monday 2 March 2015. The European Haemophilia Consortium (EHC) held its 24th Round Table on 'Product Related risk fo...

UPCOMING EVENTS

APR 16	World Haemophili 2015 Event
JUN 15	Round Table on T and Procurement
JUL 03	Youth Workshop:

News

In this section visitors will find all the **EHC News** from the home page as well as an archive of past news stories. Visitors can also access current and past issues of the **EHC Newsletter**. On the side of the page, you will also see the feed of the **Haemophilia News Facebook page**.

HOME ABOUT EHC BLEEDING DISORDERS AREAS OF WORK EVENTS NEWS LIBRARY CONTACT

EHC Newsletter

HOME > Archive for EHC Newsletter

EHC News

EHC Newsletter

EHC Newsletter – December 2014

EHC Newsletter – December 2014 In this issue: Message from the President and the CEO pg 02 – Joint Message from the President and the CEO EHC News pg 06 - EHC 2014 Conference...

READ MORE

January 5, 2015 at 10:08 am

EHC



IN THE MEDIA

Haemophilia News **Haemophilia News** 4 days ago

UK: Penrose inquiry: David Cameron apologises over infected blood

Penrose blood contamination inquiry report published [bbc.com](#)

The findings of Lord Penrose's inquiry into contaminated NHS blood supplies in Scotland are due to be published.

[View on Facebook](#) • Share

EHC Newsletter – August 2014

EHC Newsletter – August 2014 President and CEO Report pg 02 – President and CEO Report EHC News pg 06 – 25 Years of the EHC: A perspective from physicians pg 12 – Official...

READ MORE

November 29, 2014 at 11:46 pm

EHC

EHC Newsletter – April 2014

EHC Newsletter April 2014 In this edition: Message from the President and the CEO pg 02 – Joint Message from the President and the CEO

Library

In the Library section, the visitor will find all documents produced by the EHC for public distributions including Annual Reports, Position Statements and Event-related documents.

Contact

Finally, anyone can contact the EHC office using the form under the **Contact Menu**.

LIBRARY **CONTACT**

Annual Reports

Round Table Documents

Position Statements

EHC conference: Is your country ready to welcome the European bleeding disorders community?

Read to find out!

By Jordan Nedevski* and Laura Savini**



Jordan Nedevski is the EHC Vice-President Finance

bleeding disorders community and issues that may be affecting you locally. Finally, the EHC conference will provide you some revenue as 50 per cent of the conference profit is shared with the hosting National Member Organisation (NMO).

What does hosting an EHC conference involve?

Before applying for hosting the EHC conference it is important to understand that most of the organisation of the event will be done by the EHC, which is supported by a professional conference organiser. So, you do not need to refrain from applying because you are afraid to put too much work on your members and volunteers. However, some contributions will be asked of your organisation. For instance, you will be asked to get your members and volunteers involved on the two days of the conference to help out on site. This does not include processing delegates' registration but, for example, your volunteers will help delegates to find the correct meeting room, workshop room, lunch area or to provide any other type of logistical information to delegates who may need it. You will also be asked to liaise with the local haemophilia treatment centre to ask for nurses to man the treatment room. This is a place where delegates can go to get assistance for their infusion.

Furthermore, you will be asked to contribute to the development of the scientific programme. For this purpose, we will ask the President, board members and local medical experts to get involved in developing a scientific programme that will identify key issues

Each year the European Haemophilia Consortium (EHC) hosts its annual conference in a country selected by its General Assembly two years prior, meaning that in 2015, the EHC General Assembly will be selecting the hosting country for the 2017 conference. The process of putting together the bidding tender can seem daunting, however the rewards in hosting the biggest annual meeting of the European bleeding disorders patients' community are many. For example, you will be able to give your local haemophilia community exposure to the international haemophilia community and to network with fellow patients and medical and scientific experts from all over Europe. During an EHC conference, you will be bringing to your country some first-class experts and patient advocates that may help you to raise public awareness of haemophilia issues. You may also use the conference as a backdrop to support local advocacy activities and invite

politicians, government representatives, the media and other stakeholders to get them familiar with the

bleeding disorders community and issues that may be affecting you locally. Finally, the EHC conference will provide you some revenue as 50 per cent of the conference profit is shared with the hosting National Member Organisation (NMO).

The process of putting together the bidding tender can seem daunting, however the rewards in hosting the biggest annual meeting of the European bleeding disorders community are many.

in haemophilia and bleeding disorders both in Europe and in your own country.

Applying NMOs will be asked to put forward a hosting city that is easily accessible from other European countries, that is vibrant and has rich cultural and historical heritage and that has hosting capacity in terms of hotels and venues for the Conference dinner.

Traditionally, the EHC has tried to alternate its venues between Eastern and Western Europe. Although this rule is not set in stone, we do hope that many of our Eastern European members will apply for our 2017 conference

Practical details

The EHC office will send out in early summer a bidding document to all NMOs. The deadline to apply will be set to mid-August. The bidding document provides a template for planning and budgeting the proposed conference by giving concrete examples of what was done in previous years.

The EHC will ask you to maintain conference costs as low as possible so that registration fees will be affordable for all of our members and other stakeholders. The registration fees will include not only the access to the event but also lunches, coffee breaks and the Friday night dinner as well as the Saturday night Conference dinner.

The financial surplus of the conference will be shared equally between your organisation and the EHC following the event.

Travel grants

In order to allow most of the EHC members to attend the conference, the EHC provides travel grants (TG) to at least 20 of its members coming from resource-limited countries.

Each travel grant (one per country) will cover travel and accommodation expenses for one delegate per country as well as the entrance to the conference. The EHC will ask members benefiting from the travel grant to arrange their travel as early as possible (usually within the early bird times) so as to keep expenses low. The travel grant will be transferred to your organisation following the event, irrespectively on whether the delegates attends all of the scientific sessions or not, although this is strongly encouraged by the EHC.

More information

To receive more information about how to bid to host an EHC Conference, please contact Amanda Bok at the EHC office (Amanda.Bok@ehc.eu).

*Jordan Nedevski is the EHC Vice-President Finance and a member of the EHC Bulgarian NMO

**Laura Savini is the EHC Communications and Public Policy Officer

EHC 2015: Come and meet us in Belgrade!

By Jordan Nedevski and Laura Savini***

On Friday 2nd and Saturday 3rd October 2015, the EHC will be holding together with Udruženje hemofiličara Srbije (the Serbian NMO) its 28th Conference at the Crowne Plaza in Belgrade, Serbia.

Organising and Scientific Committees

This year's Conference Organising Committee is composed of:

- Mr Vladimir Illijn, President of the Serbian NMO



- Mr Brian O'Mahony, President of the EHC
- Mrs Tatjana Markovic, Vice-President of the Serbian NMO
- Mr Jordan Nedevski, Vice-President Finance of the EHC
- Mrs Amanda Bok, EHC CEO

The scientific committee advising on the content of the conference is composed of:

- Prof Dr Ivo Elezovic, University of Belgrade
- Prof Dr Dragana Janic, University of Belgrade
- Dr Danijela Mikovic, Blood Transfusion Institute of Serbia
- Prof Paul Giangrande, Chair - EHC Medical Advisory Group and University of Oxford
- Prof Flora Peyvandi, Member - EHC Medical Advisory Group and University of Milan
- Prof Angelika Batorova, Member - EHC Medical Advisory Group and University of Bratislava
- Prof Michael Makris, Member - EHC Medical Advisory Group and University of Sheffield
- Brian O'Mahony, EHC President
- Radoslaw Kaczmarek, EHC Steering Committee Member and PhD candidate at the Ludwik Hirszfeld Institute of Immunology and Experimental Therapy in Wroclaw

Programme

This year's programme will focus on the following topics:

- Haemophilia care in Serbia
- Gene Therapy
- Women and bleeding disorders
- Family planning
- Complications in Haemophilia
- Inhibitors
- Long-acting coagulation factor concentrates

The event will also run in parallel two workshops on the Friday afternoon, one of which will be aimed at bringing together women in the bleeding disorders community to start a European conversation (see article pg 23). You can find the full programme on pg 48. As always the conference will be held in English and simultaneously translated into Russian.

Posters

The conference will continue its pilot initiative on Poster presentations started during last year's conference. This initiative will give NMOs and researchers the opportunity to interact and present their new and innovative work-in-progress and to receive feedback in an informal setting. This initiative aims to give an opportunity to present preliminary findings about on-going research and projects in the haemophilia community. The deadline for application is 3rd August.

Early birds and registrations

In order to comply with European best practices the EHC has changed its categories of registrations as of last year. Healthcare professionals and representatives from the pharmaceutical industry will now be able to choose between a simple registration that includes access to the conference and the Friday night dinner and a registration that also includes the Saturday night Conference dinner. EHC

Members' registration category includes access to the conference as well as both Friday and Saturday night Conference dinners. Early bird registrations close on 24 July and we encourage as many of those wishing to attend to register early to benefit from the most favourable registrations rates.

More information

For additional information and to register, please visit the [conference website](#).

*Jordan Nedevski is the EHC Vice-President Finance and a member of the EHC Bulgarian NMO

**Laura Savini is the EHC Communications and Public Policy Officer

Pilot Leadership Conference: Building capacity and strengthening European intergenerational dialogue amongst EHC members

By Jordan Nedevski* and Laura Savini**

The EHC is organising for the first time this year a pilot Leadership Conference ahead of its Annual Conference in Belgrade. This Leadership Conference will start on Wednesday 30 September in the evening and finish on Friday 2 September at lunchtime.

Objectives of the Leadership Conference

One of the main requests from the EHC membership was to provide its members with a platform to discuss common issues and share best practices. Although EHC members meet once a year during its Annual Conference and also attend various workshops and Round Tables throughout the year, it was felt by some that all these events did not allow for enough time for conversations and sharing of everyday issues. This is mainly because each event has usually a very tight programme on a very specific issue addressed by the event.

The objective of the Leadership Conference will be to provide EHC members with enough time to learn from each other and to benefit from each other's strengths, experiences and best practices.

The objective of the Leadership Conference will be to be as interactive as possible and to provide EHC members with enough time to learn from each other and to benefit from each other's strengths, experiences and best practices. The event also aims to bring together different types of members and will specifically invite three categories: established leaders, young leaders and NMO staff taking care of day-to-day NMO activities

Programme

For this first edition, the organising committee of the Leadership Conference has selected three topics:

- Planting seeds: governance and planning. This will cover organisational matters, strategic and succession planning.
- Fundraising and financing. This session will build on the findings of the EHC survey on fundraising and financing that was carried out by the EHC in 2014 and of which the initial findings were presented during a workshop in Belfast in 2014.
- Tenders and Procurement. This session will look at the results of the 2014 EHC survey and involve participants in interactive exercises.

Travel and Accommodation

The EHC will cover travel expenses to Belgrade for all participants. Furthermore, it will cover accommodation for staff members and young leaders for the duration of the Leadership Conference.

Registrations

Registrations to the Leadership Conference are open until 30 June.

More information

For more information, please contact the EHC office at +3228932470 .

*Jordan Nedevski is the EHC Vice-President Finance and a member of the EHC Bulgarian NMO

**Laura Savini is the EHC Communications and Public Policy Officer

French Women's Committee to organise workshop for women at EHC Conference

By Yannick Colle, responsible for the Women's Committee for l'Association Française des Hémophiles (AFH – the French EHC National Member Organisation - NMO)

Since 2004, the Women's Committee of the French NMO has organised meetings and information sessions for women in the bleeding disorders community, be it partners, mothers, sisters or other women related to or sharing their lives with people with bleeding disorders. We have noticed that living with a bleeding disorder has many repercussions on the personal life of those living with these disorders, in particular on their family life and personal choices. We believe that it is important for women to have a platform where they can exchange and share experiences. Often, there is not enough information and women tend to feel isolated.

This led us to approach the European Haemophilia Consortium (EHC) with a proposal to develop further activities aimed at women in the European bleeding disorders community. I am delighted to inform you that the first such activity will take place this year as we will hold a workshop titled '*Women in the bleeding disorders community: Starting a European conversation,*' during the next EHC Annual Conference in Belgrade.

The purpose of the workshop is to provide an opportunity for women already involved in activities for women in their NMOs and with the EHC to share experiences on what they have done. At the same time, it will also be an occasion to develop new activities depending on the EHC members' needs. We want to exchange, build and consolidate a European network.

The purpose of the workshop is to provide an opportunity for women already involved in activities for women in their NMOs and with the EHC to share experiences on what they have done.



Yannick Colle is responsible for the Women's Committee of the EHC French NMO (photo courtesy of Yannick Colle)

It is our hope that through this workshop, countries that do not yet have activities aimed at women can learn and get inspired from this initiative and replicate these activities

in their own countries. We have already had this type of exchange with Portugal when two members of the EHC Portuguese NMO attended the French National Congress in 2014.

If your NMO has activities aimed at women in the bleeding disorders community or if you are looking at ways to create such activities, then this workshop is for you. Join us at the EHC Conference in Belgrade on Friday 3 October 2015 from 3.30 pm to 5.00 pm. We are looking forward to welcoming many of you in there!

The Womens' Workshop will be moderated by Yannick Colle (French NMO). Panellists will include Evelyn Grimberg (Dutch NMO), Helene Døscher (Norwegian NMO) and Christina Burgess (UK NMO).

NMO News

NMO Profile: Suomen Hemofiliayhdistys (SHY) – EHC Finnish National Member Organisation

Katja Peltoniemi interviewed by Laura Savini***

Katja Peltoniemi is the Vice-President of the Suomen Hemofiliayhdistys (SHY), the Finnish National Member Organisation (NMO) of the EHC. She talks to Laura Savini about the origins of the association, its current and future activities, and its challenges.

Katja has been volunteering with EHC's Finnish National Member Organisation, *Suomen Hemofiliayhdistys* (SHY), for 16 years. She served on the SHY board for one year before becoming its Vice-President two years ago. A mom of two boys with haemophilia, Katja found being a member of the association particularly useful. As haemophilia was not something that ran in her family, she was quite new to the community and becoming involved in the NMO helped her learn how to deal with



SHY Board Members. Top row from left to right: Mai-Lis Konnos, Katja Peltoniemi (Vice-President), Hannele Kareranta. Bottom row from left to right Meri Korpilahti, Arja Heikkilä (President), Hannu Haaranen (Treasurer). Dr Elina Armstrong and Mrs Vesa Rainne are also on the board but not depicted on the picture (photo courtesy of SHY)

the condition.

The Finnish NMO was established in 1969 following a visit from Mr Frank Schnabel, founder of the World Federation of Hemophilia (WFH), when he was on his way to Moscow for a haematology congress. Mr Schnabel visited Prof Eero Ikkala, one of the main forces behind the creation of SHY. In November 1969, Prof H.R. Nevanlinna, Prof Eero Ikkala, Prof Gunnar Myllylä and 59 people affected by bleeding disorders established SHY in the headquarters of the Finnish Red Cross Blood Service in Helsinki. Back in the 1960s, the Red Cross Blood Service was the only organisation that had the capacity and expertise to diagnose patients with bleeding disorders in Finland. Today, this organisation still performs laboratory work, although ten years ago the Helsinki University Hospital established a laboratory with capacity and expertise to diagnose bleeding disorders.

In the early days of SHY, the main objective was to advocate for access to treatment for all patients. Finland has always been influenced by its neighbour, Sweden, and it has followed the Swedish



*SHY organises regularly Members' weekends with outdoors activities
(photo courtesy of SHY)*

the beginning home treatment was used primarily for on demand treatment. By the end of 1980s most children used prophylaxis and immune tolerance induction (ITI) was also used for children with inhibitors. Currently haemophilia care in Finland is organised by one main comprehensive care centre located in the Helsinki University Hospital and four other University Hospitals (Turku, Tampere, Kuopio and Oulu). The Finnish Red Cross Blood Service is still an active service provider when it comes to consultation on how to treat people with bleeding disorders. At the moment, there is no central patient registry, although talks to establish one have been ongoing for a while. It is estimated that at the moment there are approximately 214 people with severe haemophilia A and B and 489 people with von Willebrand Disease¹².

SHY has approximately 700 members composed of patients, their families, friends and anyone with an interest in bleeding disorders. SHY has a board of eight volunteers (including a medical doctor) and a part-time secretary. The activities of SHY have the primary objective to provide social support to its members. This is done through the organisations of many events aimed at the different types of members such as families, seniors, women, children and teenagers. SHY also organises two annual meetings aimed at all of its membership: an annual meeting and an election meeting, where matters related to the management of the association such as approval of finances and elections are carried out. The next annual meeting will be held in April and it will feature a medical/scientific lecture, which this year will focus on inhibitors in both adults and children. The event will also feature a session for younger members on the impact that living with a bleeding disorder has on career choices. For this purpose, SHY will invite a haematologist and student counsellor to speak with its younger members. Besides these annual meetings, SHY organises a series of other events such as winter sports weekends, summer camps, women's weekends, an all-boy/men weekend and family weekends. SHY has a very busy programme, especially considering that all board members are volunteers with families and full-time jobs.

In Katja's opinion, it is very important for SHY to provide a platform and social outlet where people can meet and share similar experiences. This is currently the main priority of SHY. In fact, with a good

protocols for haemophilia treatment quite closely. Although Finland did not adopt prophylaxis as quickly as Sweden, it took it on board quite soon after Sweden did. Home treatment in Finland started as early as 1974 when the Finnish Red Cross Blood Service held a home treatment learning course for patients with bleeding disorders and their families. At



SHY Members during the NMO activities (photo courtesy of SHY)

¹² 2014 figures gathered from the Social Insurance Institution of Finland's statistics on home treatment.

level of treatment and access to care there is no real need for SHY to be politically active.



SHY members during the NMO activities (photo courtesy of SHY)

at all and that no actions are needed.

For Katja, SHY has brought some real help in terms of how to deal with the disorder and to let her boys know that they are not alone in being affected by haemophilia. She has made many friends and found lots of support through the network provided by SHY. She now hopes that she can give back some support and share knowledge and experience with other members.

**Katja Peltoniemi is the Vice-President of the Suomen Hemofiliayhdistys (SHY), the Finnish National Member Organisation (NMO)*

***Laura Savini is the EHC Communications and Public Policy Officer*

Italy elects new President: Interview with Cristina Cassone

Cristina Cassone interviewed by Laura Savini***



*Cristina Cassone is the newly elected President of FEDEMO, the EHC Italian NMO
(photo courtesy of FEDEMO)*

At the end of 2014 the Federazione delle Associazioni Emofilici ONLUS (FEDEMO), the EHC's Italian National Member Organisation (NMO), elected a new President, Mrs Cristina Cassone, for 2015-2017. Laura Savini speaks to her about her background and priorities for FEDEMO.

Cristina is the mom of a child with haemophilia and she has been extremely engaged in the haemophilia community since 2008, when she founded the L'Associazione dei Bambini Coagulopatici ed Emofilici (ABCE), the local haemophilia association for children covering the Apulia region (in the South-East of Italy).

Cristina played a key role in many projects at both the local and national level towards the improvement of access to treatment for people with bleeding disorders. For instance, she worked towards the creation of a local network of haemophilia treatment centres in the

Apulia region as well as towards the reform of the regional legislation on home treatment. In 2010, the network was successfully established

However, Katja stresses that despite not being politically active at the moment, SHY always keeps an eye on ongoing issues that may affect access to treatment.

SHY is also looking into ways to build collaboration with the Estonian Haemophilia Association. In fact, Helsinki and Tallin University Hospitals are currently part of a WFH twinning programme and the two patients association are looking at ways to collaborate.

Due to the good treatment situation in Finland, one of the main challenges faced by SHY is how to attract and retain volunteers, in particular younger ones as they feel that they do not have any problems

and the new legislation for home treatment was implemented. In 2011, she collaborated with the local Haemophilia Treatment Centre in Bari (the capital city of the Apulia region) to organise the first conference on children with haemophilia and the role of families and associations. Her work includes many more activities, such as training on self-infusion and information sessions for healthcare professionals working in Hospital Emergency Units on how to deal with people with bleeding disorders. She also collaborated on the accreditation of the local haemophilia treatment centre by the regional coordination centre for rare diseases. Cristina further collaborated on various projects with FEDEMO at a national level on women in the bleeding disorders community (Progetto Finestra Rosa) and on how to care for people with bleeding disorders in Hospital Emergency Units (Progetto Safe Factor).

Although only active in the Italian bleeding disorders community for a few years, Cristina showed through her dedication and entrepreneurial spirit a real commitment to the bleeding disorders community, something motivated by her personal experience as a mother of a child with haemophilia. It only seemed natural for her to continue her good work on a national level. In her election campaign, Cristina underscored the importance of tightening the relations between FEDEMO and its regional members and the families in the bleeding disorders community. To accomplish this, FEDEMO has asked regional associations to designate a liaison person for FEDEMO's activities and has surveyed its members to find out what the most pressing issues are for each regional association. FEDEMO will furthermore modernise its communication tools, including its website and newsletter.

Another objective will be to maintain and strengthen FEDEMO relationship with government agencies and officials. It will maintain its partnership with Fondazione Paracelso, a foundation with the objective of providing social solidarity among patients with haemophilia. Finally, FEDEMO will focus on the promotion of sports and active living for people with bleeding disorders, as well as the training of younger volunteers to become the leaders of tomorrow.

It is with this challenging programs that Cristina started her Presidency in December together with a new board. The EHC wishes her and the entire FEDEMO board a successful and fruitful term.

**Cristina Cassone is the President of the Federazione delle Associazioni Emofiliici ONLUS (FEDEMO), the EHC's Italian National Member Organization (NMO)*

***Laura Savini is the EHC Communications and Public Policy Officer*

Portuguese First National Congress: Leira, 27-29 November 2014

By Miguel Crato, President of APH, Associação Portuguesa de Hemofilia, the EHC Portuguese National Member Organisation (NM O).

APH, Associação Portuguesa de Hemofilia organized last November the first National Congress of Hemophilia, in Leiria, Portugal.

This event was clearly a need for our community, not only for the patients and their families, but also for the clinicians, nurses and everyone who deals with haemophilia and other bleeding disorders. Information, training and sharing were the main goals for the First National Congress and they were fulfilled with great delight for all the 206 participants.



Delegates of the first APH Congress (photo courtesy of APH)

During the weekend we had a mix of workshops and scientific panels. We tried to reach the maximum areas of interest for people with haemophilia.

In the opening ceremony, after I gave the welcome address to all participants, we had a group of testimonies aimed at representing all the different parts of the haemophilia community such as: a physician, a nurse, a child with haemophilia, a woman with a bleeding disorder, a young man with haemophilia and Olivia Romero-Lux, Member of the European Haemophilia Consortium (EHC) Steering Committee, as EHC representative.

In the workshops we covered areas such as haemophilia and communication, ageing, genetics,



Panellists during the Medical Session (photo courtesy of APH)

children and haemophilia, and blood disorders in women. The medical panels included topics such as inhibitors, hepatitis C, new technologies and long acting products, arthropathy in haemophilia and pain management in haemophilia.

Healthcare professionals from the five major hospitals in Portugal attended the conference, gave presentations and took part in the workshops. It was very satisfactory to watch how physicians, nurses and patients worked together in a very dynamic way.



Delegates of the APH 'Mini-Congress' (photo courtesy of APH)

We had the honour, too, to have a session dedicated to haemophilia and sports with the presence of Alex Dowsett who gave his testimony as a young person that is completely able to enter in a professional sport competition and how he and his family can cope with that.

Simultaneously, during the Congress we had the 'Mini-Congress' addressed to children from four to 14. They were involved in a series of activities aiming to provide them with the basics about haemophilia in a fun and uncomplicated way. In the end they presented a theatrical show their work during the weekend to all participants. All the children older than 14 were encouraged to attend the normal sessions.

Our Congress was an opportunity to also involve our youth committee in the organisation of such a great event. They took care of all the logistical support for the Congress and it worked very nicely.

In conclusion, we fulfilled our objectives and the Congress was an excellent opportunity for everybody to get involved and receive important information about haemophilia and other bleeding disorders.

Rise like a phoenix

By Olivia Romero Lux, EHC Steering Committee Member and Member of the EHC French NMO

EHC Board Member, Olivia Romero-Lux reports on her attendance at the first congress of the EHC Portuguese Member, Associação Portuguesa de Hemofilia (APH).

Establishing a national haemophilia society is not an easy task, but keeping it alive, active and kicking is even more difficult. Every National Member Organisation (NMO) has gone and will go through ups and downs. But in order to serve the community of haemophiliacs at a national level in the long run, it is important for a NMO to meet with a special person, who in turn, will gather a team of skilled volunteers and staff members to meet the community's expectations.

When I was invited to attend the Annual Congress of the Portuguese NMO last November, I did not realise that I was about to experience one of those precious moments when you feel that a specific haemophilia society has reached a turning point.

Indeed, after being welcomed by very enthusiastic staff members at the NMO headquarters, I arrived at the Congress venue and discovered that the Portuguese society was holding its first general

conference in years. A few years back, the APH went through a terrible tragedy that had torn its foundation apart.

Now, in 2014, Miguel Crato, the President of the APH, and his team have managed to pull things back together and to organise a terrific Congress with a top-notch scientific programme and a whole team of volunteers ready to serve the community.



The APH Board. Miguel Crato is fourth from the left (photo courtesy of APH)

Why write about this? What is my point in telling you about this Congress?

Well, the EHC represents altogether 45 NMOs, all so different one from the other. In most cases, the NMOs run thanks to a charismatic and skilled leader and a few very active members but sometimes, the NMO goes through a temporary crisis and it takes some time to get it back up and running.

If you feel that your NMO is going through difficult times, do not give up, gather up a good team of skilled and motivated people. If they need help, have them trained, assign them specific missions according to theirs skills and their availability, rise like a phoenix and get back into motion!

Newly Diagnosed Weekend

By Christina Burgess, Head of Membership and Planning, the Haemophilia Society UK

The EHC UK National Member Organisation (NMO) has developed a programme aimed to support families with a recently diagnosed child with haemophilia. Christina Burgess, membership officer for the Haemophilia Society UK reports on the experiences gained from these events.

2015 started with two fantastic retreats for families with a newly diagnosed child in the UK. Thirty-two (32) families with newly diagnosed young children plus their siblings (a total of nearly 80 adults and 80 children and young people) attended free weekends at popular family holiday locations, CentreParcs, and were able to enjoy fantastic facilities, as well as meet others who really understand the challenges, fears, emotions and isolation a diagnosis can bring. We provided a daytime crèche so that parents would be able to focus on the sessions. Using the crèche in itself was in some cases the very first time a mother had left her child in the care of strangers. This first step, even if only for 20 minutes at a time, helped build parents' confidence for when their child reaches nursery school age and beyond.

We know a diagnosis affects the whole family, so siblings were also invited to attend. Many older brothers and sisters were quite involved in looking after their newly diagnosed sibling and it was clear the impact their siblings' disorders must have on their own lives. Several of these young people have signed up to attend our teen weekend in the spring, so they can meet again and really feel part of the community.



A dad and his son during the Newly Diagnosed Weekend (photo courtesy of the Haemophilia Society UK)

During the weekend, we also had a group of highly experienced volunteers made up of haemophilia nurses, paediatric physiotherapists, haemophilia doctors and paediatricians and two of our Youth Ambassadors, Luke Pembroke and Rob Barnard. Both are 20 and have severe haemophilia. Luke is studying biology at university and Rob is training to be a physiotherapist.

Sessions ranged from 'Newly diagnosed, what does this mean for you?' to separate sessions for mums and dads on 'What do you think and what do you think they think?' This gave everyone, but particularly dads, the opportunity to open up about feelings they sometimes suppressed.

It became clear that quite a few parents, prior to their child being properly diagnosed, had been wrongly suspected of child abuse. It was very helpful for them to talk this traumatic episode through in a safe environment and it is useful to make other parents aware that they might encounter this in the future and how best to deal with it.

Many parents were worried about their child's potential to take part in sport in the future. They were concerned that if their child could not take part with classmates at school, this could possibly lead them to being singled out and maybe even bullied? Luke and Rob, were able very eloquently to dispel this concern. The sessions culminated with them sharing how they had coped both through their school and college life and their social life with non-affected friends and peers. They also each gave a demonstration of their treatment and provided parents with tips on *hitting a vein* and the opportunity to familiarise themselves with needles and equipment.

We thought we would share some of the feedback from parents:

"I cannot tell you how valuable that weekend was for us, as a family, coping with haemophilia and we will be forever grateful to you for organising such a superb weekend."

"It was such an informative, emotional and inspirational weekend. We found it very helpful! It was just great to hear the experiences of other parents with haemophiliac/bleeding disorders children. We learnt so much from this and the talks and presentations. It was great to have the chance to ask any questions and deal with any worries we have and just to know that everyone else has the same ones. Often listening to the other parents, it was like they were taking the words out of my mouth."

"I just feel like a massive weight has been lifted, which I never knew was actually there. I think as a parent with a child of any



Participants during the Newly Diagnosed Weekend (photo courtesy of the Haemophilia Society UK)

condition you get fixated on them as they are your world and you then forget about yourself and how it is actually affecting you. It was a very emotional weekend, but in a positive way. When people have asked about it, I have said it was like counselling - I mean that in such a positive light. To be in a room with other people who actually 'get it' just made the world of difference and we will be forever grateful for the experience The Haemophilia Society has allowed us to take part in."

If you would like to find out more about the weekends do contact me at christina@haemophilia.org.uk.

The All Party Parliamentary Group on Haemophilia and Contaminated Blood (APPG)

By Liz Carroll, Chief Executive Officer, the Haemophilia Society UK



Member of Parliament Mrs Diana Johnson MP and Mrs Liz Carroll with the APPG Report (photo courtesy of the Haemophilia Society UK)

Liz Carroll, CEO of the Haemophilia Society UK writes on the work carried out by The All Party Parliamentary Group on Haemophilia and Contaminated Blood (APPG).

At my first meeting with The All Party Parliamentary Group on Haemophilia and Contaminated Blood (APPG) early in 2014, I suggested they should look at undertaking a project that could have a really positive impact on our community, as well as raising the profile of the APPG.

All Party Parliamentary Groups are informal cross-party groups that have no official status within Parliament, and are run by and for Members of the Commons and Lords. However, despite not having a formal status, APPGs can be very influential in bringing issues to the attention of Members of the Parliament (MPs) and Lords and can support and influence government activity.

Both the charity and MPs frequently receive calls from people who were affected by the contaminated blood tragedy in the 1970s and 80s and who are struggling to find their way through the complex system of financial support set-up by the government in the UK.

In light of this, the APPG decided to undertake an inquiry into the current support for those affected by the contaminated blood scandal in the UK. As the secretariat for the APPG, the Haemophilia Society UK coordinated and funded the inquiry. At the same time we have been working with Alistair Burt MP who is working with the Prime Minister's office on a possible future package of support for those affected.

A survey was sent to all registrants of the five organisations set up to support those affected as well as the survey being advertised on social media, via the Societies' Facebook and twitter feeds. The survey had two sections, one on their experiences of the current systems and one on their hopes and requirements for future support. The APPG was responsible for part one, Alistair Burt MP for part



UK Haemophilia Society Trustee Mr Alan Burgess (photo courtesy of the Haemophilia Society UK)

two. The survey was kindly hosted pro bono by YouGov¹³ and 961 respondents completed the survey.

The survey was analysed by an independent researcher and additional qualitative data added to the findings to develop a full report. The report can be found on [our website](#).

The report was launched on 14 January 2015 in Parliament and was attended by members of the House of Lords, MPs and members of the affected community. The co-chairs of the APPG Diana Johnson MP and Jason McCartney MP both spoke about why the report was so important and then Alan Burgess, a Haemophilia Society trustee spoke about his experiences as a beneficiary of the current system and his experience of being a trustee of the McFarlane Trust (nominated by The Haemophilia Society). Neither were positive experiences.

The following day Alistair Burt MP led a Back Bench Debate in the House of Commons MPs debated the following motion:

'That this House supports a further review of the circumstances surrounding the passing of infection via blood products to those with haemophilia and others during the 1970s and 1980s; notes the recent report from the All Party Parliamentary Group on Haemophilia and Contaminated Blood into the support arrangements provided for those who contracted blood-borne viruses as a result; also notes that the Penrose Inquiry into these events will shortly be publishing its findings in Scotland [at the time of this statement the report was published]; further notes that those who contracted viruses and their partners and dependants continue to be profoundly affected by what happened;

therefore welcomes the Prime Minister's commitment to look again at this issue; and calls on the Government to respond positively to the APPG report and engage actively with those affected with a view to seeking closure to these long standing events.'



Members of Parliament Mr Alistair Burt and Mrs Diana Johnson co-chairs of the APPG (photo courtesy of the Haemophilia Society UK)

During the debate a large number of MPs spoke, recounting devastating stories of the experiences of their constituency members, watched by a large number of people who have been personally affected by the tragedy. It was a moving and powerful debate.

Following the two days of activity we were able to secure a great deal of media attention with a range of TV, radio and newspaper interviews and many campaigners were able to share their experiences.

We are working hard to maintain focus on the issues our community face and hope to bring this to a positive conclusion soon. The upcoming General Election in the UK may delay things a bit, but we are determined not to let this opportunity pass.

Post-article note:

The Enquiry conducted by Lord Penrose on the contamination with HIV and hepatitis C of thousands of people in Scotland through blood and blood products was published on 25 March 2015. The report

¹³ An online market research company.

took six years of preparations and looked at hundreds of thousands of documents, as well as hundreds of testimonies from those who were contaminated, including many people with bleeding disorders. The report only provides one recommendation, which is that the Scottish government should offer hepatitis C tests to people having received a blood transfusion prior to 1991 and who have not yet been tested for hepatitis C.

In response to the publication of the report, UK Prime Minister David Cameron gave a public apology to those affected by what has been called the worst treatment disaster in the history of the UK National Health Service (NHS) and stated that an interim fund of £25 million will be set up to offer compensation to those affected by the contamination.

The APPG also issued a public response to the publication of the report:

The Contaminated Blood Scandal was the biggest disaster in NHS history. It was right that there was a judicial inquiry. We wish to express our appreciation for the commitment Lord Penrose has given, and hope he recovers from his illness. Given that the report extends to five volumes and over 1800 pages it will take some time to digest, but we share the surprise of many of those affected that the Inquiry only made one recommendation.



Today we should remember all those who contracted HIV and Hepatitis C and their families; for them this is not a historic issue but an ongoing tragedy, which continues to have a devastating impact on their lives. Lord Penrose was right to say this was the stuff of nightmares.

The apology from the Prime Minister on behalf of the UK Government is a significant moment in a long struggle for recognition of the scale of this tragedy, and we welcome this. What we need now is a proper system for supporting and compensating all those affected. The APPG Inquiry last year showed that the current support system is simply not meeting the needs of those it is meant to help and is not fit for purpose.

There were some positive steps today: we welcome the announcement of £25m for transitional funding and we welcome the commitment from both party leaders that whoever forms the next Government they will provide a permanent settlement for all those affected. This is not the end of the matter further steps need to be taken in keeping with MPs previous recommendations. We will continue to support those affected, and those who have been left behind. After decades of injustice a lasting settlement is long overdue.

For more information and an update on the follow-up actions to the Penrose Enquiry, we invite you to visit the Haemophilia Society UK website: <http://www.haemophilia.org.uk>

Special St. Camillus Prize for Bogdan Gajewski, President of the Polish Hemophilia Society

By Adam Sumera, Vice-President of Polskie Stowarzyszenie Chorych na Hemofilię the EHC Polish National Member Organisation.

Polskie Stowarzyszenie Chorych na Hemofilię's President, Mr Bogdan Gajewski was awarded with the St Camillus Award. Adam Sumera, Vice-President of the EHC Polish National Member Organisation (NMO) reports.

"This is to honour the fact that serving your fellow man has become the highest value in your everyday life as well as your total commitment to the struggle for a better tomorrow for yourself and other patients." This was the St. Camillus Award board's statement accompanying Bogdan Gajewski's

award of the Special St Camillus Prize for extraordinary achievements in helping the sick.



From left to right: Mr Bogdan Gajewski, Polish Prime Minister Mrs Ewa Kopacz, Father Arkadiusz Nowak (photo courtesy of the EHC Polish NMO)

The St Camillus Award is granted every year on 11 February, the World Day of the Sick, for special achievements in providing care for the sick. The award was established in 2007 on the initiative of the St Camillus' Order and the Institute of the Patient's Rights and Health Education. The main idea of the award is to promote people and institutions contributing to building acceptance for, and solidarity with, the ailing and their families. The award is "*a voice for helping those who are unable to publicly demand the observance of their basic rights.*"

The award was handed out by the Prime Minister Ewa Kopacz. Upon receiving the award, Bogdan Gajewski said, *"Prime Minister, I would like to thank you for introducing the prophylactic treatment for children with haemophilia at the time when you were the Minister of Health. Before that, children with haemophilia spent a lot of their time in hospitals. Now, thanks to the prophylaxis, they can attend kindergartens and schools and live the same lives as their healthy peers. Now we are waiting for all the postulates set in the National Haemophilia Treatment Programme to be fulfilled by Minister Bartosz Arlukowicz, who is also with us here."*

This is the second decoration for Bogdan Gajewski honouring his considerable achievements for people with haemophilia in Poland. In 2010, following his successful efforts to introduce prophylaxis for Polish children with haemophilia, he was awarded the Order of the Smile, the only child-nominated award in the world recognised by the United Nations.



Mr Bogdan Gajewski with the award (photo courtesy of the EHC Polish NMO)

Feature Articles

Veggies for treatment of haemophilia

By Prof Roland Herzog* and Prof Henry Daniell**

We recently read in the news about the ‘lettuce pill’ as a potential way for people affected by inhibitors to continue taking coagulation factor concentrate without the negative side effects. We asked the researchers behind this innovative method to give us some further explanations about how this pill works.

Inhibitor formation is a major problem in the treatment of haemophilia and a big scare for any patient with this bleeding disorder. Although there are bypassing agents that allow doctors to restore blood clotting in patients who develop such antibodies, treatment is much more complicated in this circumstance. It is estimated that 20 to 30 per cent of patients with severe haemophilia A develop inhibitors against factor VIII during replacement therapy. This risk is further elevated depending on the genetic mutation, ethnic background, and other factors. Eradication of the inhibitor using current protocols requires daily infusion of factor and can take months to more than a year. In addition, this immune tolerance induction does not work in all patients. Inhibitors against factor IX (in haemophilia B patients) are comparatively rare, but are often even more difficult to eliminate, as patients may develop severe allergic reactions to factor IX or experience damage to their kidneys. There has been much recent progress in laboratory studies to develop protocols that prophylactically induce immune tolerance to coagulation factors, so that inhibitors will not form in the first place. Although this



Harvesting the lettuce in Daniell's laboratory (photo courtesy of Prof Henri Daniell)

concept is interesting, use of immune suppressive drugs or genetic modifications pose other risks that have not been found acceptable in young children. On the other hand, if one could develop an oral tolerance, one could simply eat or drink factor VIII or IX to tolerise the immune system.

The concept of oral tolerance for haemophilia is therefore much more appealing, but has not been easy to accomplish. Large amounts of clotting factor are needed to make it through the harsh environment of the stomach and to the immune system in the gut before being degraded. This is where we sought to develop an alternative method that we believed has a better chance of getting the clotting factors to the intestinal immune system.

The Daniell laboratory has pioneered expression of several human proteins within plant cells for treatment of a number of human diseases. Because the plant cell wall protects these clotting factor proteins through '*bio-encapsulation*,' they survive the harsh acidic environment and digestive enzymes. Human enzymes are incapable of digesting plant cell wall. However, when plant cells travel



*Daniell's lettuce capsules with blood clotting factor
(photo courtesy of Prof Henri Daniell)*

further in the digestive tract, healthy microbes that colonize the gut digest plant cell walls, thereby releasing human therapeutic proteins. Therefore, when fused with suitable cell penetrating tags, human blood clotting factors expressed in plant cells are efficiently delivered to the immune system. In the Herzog laboratory, repeated delivery of blood clotting factors made in plant cells induced tolerance to both factor VIII and IX, so that no anaphylaxis occurs and inhibitors are not formed or at least produced at much lower levels. Our most recent studies uncovered the mechanism of how this promising protocol for oral tolerance induction prevents immune responses against clotting factors in the treatment of haemophilia.

For more details, click on web links below:

- [Scientific American](#)
- [Nature](#)
- [BBC](#)
- [Science feature](#)
- [Blood commentary – Take a leaf from the book of oral tolerance](#)
- [Blood article](#)

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**Prof. Henry Daniell works at the Department of Biochemistry, School of Dental Medicine, University of Pennsylvania, Philadelphia.

Highlights from the International Plasma Protein Congress 2015

By Radek Kaczmarek, EHC Steering Committee member and member of the EHC Polish NMO

EHC Steering Committee Member, Mr Radoslaw Kaczmarek, attended the 21st International Plasma Protein Congress (IPPC) in Rome, where he moderated a session together with other patients' advocates. Here is the report of the presentations and discussions held during the event.

The 21st IPPC took place in the Eternal City, Rome. The meeting was divided into eight sessions covering a wide range of aspects related to production of and treatment with plasma-derived medicinal products (PDMPs) and their recombinant equivalents, from global use and needs, to access to care to emerging issues.

The Opening Session kicked off with a panel featuring Dr Paolo Marcucci (Chairman and Managing Director at Kedrion Group), Dr Giuliano Grazzini (Director Centro Nazionale Sangue – the Italian National Institute for blood), Dr Lorenzo Montrasio (Head of Biological Medicinal Products Unit at



The patient panel during IPPC 2015. Mr Radoslaw Kaczmarek is the first from the left (photo courtesy of PPTA)

AIFA, the Italian Medicines Agency), and Mr Patrick Robert (President of the Marketing Research Bureau). Dr Marcucci, Chairman of the Global Board of Directors of the Plasma Protein Therapeutics Association (PPTA) highlighted the Association's mission to promote the availability of and access to safe and effective plasma protein therapies for all patients. Next, Drs Grazzini and

Montrasio described medical and legal aspects as well as the regulatory framework of plasma

collection, processing and use in Italy. Dr Robert addressed in the final talk the concept of self-sufficiency in PDMPs. There is no simple answer to the question of how self-sufficient we are globally as each country is different. Moreover, the treatment landscape dynamically changes with the emergence of new innovative products that replace some PDMPs; which makes the concept of plasma self-sufficiency obsolete in the context of some diseases, particularly bleeding disorders. On the other hand, the demand for plasma-derived immunoglobulins may increase if new indications for use of these drugs appear, thus making the problem of self-sufficiency as important as ever. The session ended with announcing Prof Ann Gardulf from the Karolinska Institute in Sweden and the winner of the 2015 Hilfenhaus Award, named in the honour of Dr Joachim Hilfenhaus, a notable virologist. Prof Gardulf has focused her career as a leading researcher in the administration of subcutaneous immunoglobulin. She has dedicated her life to improving the lives of patients with primary immunodeficiencies who live with lifelong treatment that is usually taken on a weekly basis.

The Regulatory Panel featured various perspectives, including Dr Jay Epstein (Director of the Office for Blood Research and Review at the United States' Food and Drug Administration – FDA) who gave an overview of the FDA policies that affect plasma and plasma protein therapies, Dr Anneliese Hilger (Head of Sections Coagulation Products at the Paul-Ehrlich-Institut) who gave a perspective on the European Medicines Agency (EMA) regulatory landscape, Dr Tommaso Paoli from Kedrion on epidemiology and industry; and Dr Micha Nübling from the World Health Organization (WHO), who spoke on the European Medicines Agency (EMA) epidemiological guideline. Dr Epstein reported that there will be a policy change on the deferral of male donors who have had sex with men (MSM). A guidance document is being developed with an implementation date in 2016. Many countries have

been revisiting their MSM policies recently and the [EHC has issued its position on this topic, which can be read here](#). Dr Nübling noted that a new epidemiology guideline will be published later this year. Two goals of that guideline include removing burdens of reporting and seeking harmonization between plasma master files by using standard window periods and incidence estimates for first-time donors.

Session 4 was entirely devoted to challenges and opportunities for patient access to care. The session was chaired by Mr Larry Warren, a long-time leader of Alpha-1-antitrypsin deficiency patients' community, and co-chaired by myself. The first speaker, Mr Brian O'Mahony, President of the EHC, spoke on the importance for physicians and patients to work together towards the common goal of access to safe, efficacious and cost-effective therapies. Mr O'Mahony lamented the yawning gap in access to clotting factor concentrates and optimal quality of care between some European countries, even within the European Union. New generations of products that are now entering or will soon enter the market may be an opportunity to improve access to safe and effective treatments in countries at the lower end of health expenditures. Speaking about the challenges, Mr O'Mahony pointed out how haemophilia care in countries that have been enjoying good quality of care for a while, may become a victim of its own success as patients organisations grow complacent. *Does high quality care mean that patient advocates can retire with relish while the young may enjoy life and leave the past behind?* The answer to this is a resounding 'no'. Patients always have to be prepared to make a strong case to defend their treatment. Finishing his talk, Mr O'Mahony shared an inspiring video of nine-year-old Adam, a young haemophiliac proudly showing himself infusing. Mrs Jose Drabwell, Chair of the International Patient Organisation for Primary Immunodeficiency (IPOPI) provided a comprehensive update of the organisation's achievements in promoting access to care. Alpha-1 representative Dr Frank Willersinn, Steering Committee Member of Alpha-1 Global is both a patient himself and a medical doctor. Dr Willersinn spoke of the difficulties in access to therapies in Europe. In addition, he noted that although access to alpha-1 proteinase inhibitor is good in most countries in the European Union, there remain several countries that do not reimburse for the treatment. The global scope of the Alpha-1 Foundation was covered by Mrs Gonny Gutierrez, Global Program Director at Alpha-1 Foundation, with an emphasis on research and diagnosis, awareness and an extensive advocacy network.

The second day of the congress included more discussions on access to care but from the perspective of Health Technology Assessments (HTA) in relation to licensing, market access, and market approval. Dr François Meyer from the French National Health Authority explained an EMA pilot program that aims to improve licensing with regard to timely access for patients.

This year the EMA also introduced the Safe and Timely Access to Medicines for Patients (STAMP) program that currently comprises 23 Member States. Dr Lieven Annemans, from the University of Gent, pointed out that despite the fact every dollar invested in health care will come back as 2 dollars to the economy, tough choices still dominate patient decisions. He called for faster HTAs, including better data assessments, harmonization, and more people training in health economics. Dr Annemans also emphasized the importance of payers, patients, and industry working together throughout the process as well as before undertaking clinical trials. Prof Albert Farrugia emphasized that it is not HTA authorities, who make decisions, but politicians. He also denounced as unacceptable that after therapies have been approved by regulatory agencies in the context of reimbursement, randomized clinical trials are being demanded. That puts haemophilia patients at unnecessary risk for joint damage.

Mr O'Mahony pointed out how haemophilia care in countries that have been enjoying good quality of care for a while, may become a victim of its own success as patients organisations grow complacent.

In the final session the audience could hear more international perspectives. Mr Cesar Garrido, a former presidential candidate for the World Federation of Haemophilia (WFH), spoke on the need to close the gap between the number of Latin American patients estimated to have haemophilia and those actually diagnosed with the disease. According to Mr Garrido, there are approximately 58,000 people living in 19 Latin American countries who have haemophilia but only 28,000 have been diagnosed. Mr Alan Chit delivered a comprehensive presentation on China covering political, regulatory and patient access to care issues. Given the 1.2 billion population there is significant potential to be a major market for plasma protein therapies. However, trade barriers exist; some of which results from a cultural belief about how plasma products should be treated. The Chinese belief system results in a lack of a stable supply, which compromises patient care. It is estimated that currently only 50 per cent of the needed plasma is collected. As far as treatment issues, it is arduous for patient groups to engage with other stakeholders because of the political make-up. Chinese authorities are extremely wary of any real or perceived external interventions.

The 2016 IPPC will be held in Barcelona.

Rare Disease Day celebration in Brussels

By Laura Savini, EHC Communications and Public Policy Officer

On 24 February, the staff of the European Haemophilia Consortium (EHC) attended the event organised by the European Rare Disease Organisation (EURORDIS) for its members and partners to celebrate Rare Disease Day, an international commemoration to raise awareness about rare diseases and the issues faced by people living with them.

The EURORDIS events are usually very well attended by representatives from the European Institutions such as the European Commission and the European Parliament and other European agencies such as the European Medicine Agency (EMA) and this year was no exception. The event is the opportunity to make the point on progress done in terms of policy and regulation supporting the diagnosis, access to treatment and recognition of the needs of people living with rare disorders. Mr Vytenis Andriukaitis, the recently appointed Commissioner for Health and Consumers, opened the event by unveiling that the European Commission will launch a new joint action on rare diseases. He also called upon all Member States to adopt national plans for rare diseases.

Member of the European Parliament (MEP) Mr Philippe De Backer followed Mr Andriukaitis by proposing the idea of developing a common purchasing mechanism for medicinal products for rare diseases in Europe. In his opinion, the diversity of reimbursement procedures and marketing mechanisms in Europe does not facilitate the rapid access to therapies for rare diseases. If industry could deal with a single counterpart it would result in better pricing and access for patients.

The event then progressed with different panels covering topics like reimbursement, clinical trial protocols, patient participation in EMA activities, and patients' advocacy in policy development. Each



Commissioner for Health, Mr Andriukaitis during the opening address of the Rare Disease Day Event (photo courtesy of EURORDIS)

panel featured a patient together with other stakeholders such as healthcare professionals, industry, regulators and payers. The importance of patient involvement in every aspect of healthcare from the design of clinical trials to the marketing of medicinal products, the delivery of healthcare and the development of policies and regulations was highlighted in each panel. In fact, patients are the primary consumers of healthcare services, products and procedures and they can provide a unique insight in what is working and what can be changed. This is the same as advocated by the European Haemophilia Consortium (EHC) during its December Round Table on National Haemophilia Councils (see EHC Newsletter 2014 vol. 3).

All sessions featured interesting discussions but a few key points are worth taking note of.

In the session covering reimbursement, Dr Ri De Ridder, the General Director of the Belgian Reimbursement Agency for Medicinal Products, praised current initiatives for increased dialogue between payers and manufacturers of medicinal products in earlier stages of product development. In his opinion, this process will help to accelerate reimbursement and get medicinal products more quickly on the market. He also noted the disparities in pricing for a same product across various European countries as prices are adapted to the perceived purchasing powers of Member States in Europe.

In the clinical trials session, panellists noted the difficulties in getting patients involved in early stages



*Mrs Miroslavljevic giving an emotional account of her experience as patient advocate
(photo courtesy of EURORDIS)*

of product development. On the one hand, barriers can come from within pharmaceutical companies where the benefits of patient involvement are often not perceived to their true value. Luckily, mentalities are changing and increasingly companies see the benefit of asking for patient's advice when, for example, designing a clinical trial. On the other hand, there is increased scrutiny on the relations between patients and the pharmaceutical industry and it was noted that there can be legal obstacles to develop successful patient-industry relationship. Furthermore, patients' image and credibility can

be damaged if patients are perceived to be too close or partial to industry. Nonetheless, the value of having patients' experts was highlighted as they can provide advice on the impact of living with certain conditions, something that is often overlooked by people developing therapies.

Mrs Nathalie Bere in charge of patients' relations at the EMA provided an overview of how patients are directly involved in the Agency's activities. Mrs Helma Gussek, a patient affected by retinitis pigmentosa¹⁴ taking part in EMA's activities gave an account of her experience. She noted that being involved in EMA's activities required a lot of preparatory work, which can be difficult for people with disabilities or for people who do not have a good command of English. She also noted that being the sole patient representative in a room full of medical, scientific and industry experts can be daunting. Another difficulty of the role was to represent a whole patient population based on a single person's experiences. Despite all of these difficulties, the experience of Mrs Gussek was ultimately a positive

¹⁴ Retinitis Pigmentosa is an inherited, degenerative eye disease that causes severe vision impairment due to the progressive degeneration of the rod photoreceptor cells in the retina.

one and she encouraged other patients to take on this role, as it is important to have patients' representation in regulatory agencies such as the EMA.

The event was concluded with the poignant testimony of Mrs Bojana Miroslavljevic, the mother of a girl with Batten Disease¹⁵. Mrs Miroslavljevic recounted the struggle she went through to find the diagnosis and provide adequate care for her daughter. The story of Mrs Miroslavljevic was both tragic and inspiring and visibly touched the entire audience moving many, including the EHC staff, to tears. Mrs Miroslavljevic's daughter, Zoya, developed signs of Batten diseases such as mental impairment, seizures, and progressive loss of sight and motor skills at an early age. Doctors in Serbia did not know the disease and therefore dismissed it as non-existent. This prompted Mrs Miroslavljevic and her husband to sell off all their possessions and travel to London to visit a specialised centre where their daughter could be diagnosed. Once Zoya was diagnosed, the family returned to Serbia but seeing Batten Disease was not included in the Serbian Disease Classification System, doctors dismissed again the disease as non-existent and the family was not able to access any medical or social care.



*Participants at the event holding hands to mark solidarity amongst rare diseases
(photo courtesy of EURORDIS)*

Once Zoya passed away Mrs Miroslavljevic, with the help of the Institute for Social Sciences & Association for Medical and Health Law of Serbia, decided to launch an advocacy campaign to change the Serbian legislation. This resulted in a new legislation 'The act on prevention and diagnosis of preventable genetic diseases' also nicknamed 'Zoya's law,' which made compulsory for Serbian health institutions to send genetic samples abroad if they could not provide a diagnosis within six months of the first visit. Additionally, families with one child living with a genetic disease are given access to prenatal testing at no cost and genetic testing is offered to other family members.

Mr Terkel Andersen, President of EURORDIS, concluded the event by stating: "*Today we have seen moving examples of not only the challenges patients and parents live with day-to-day, but of the extra courage they need and effort they make to influence policy.*"

¹⁵ Batten disease is a fatal, inherited disorder of the nervous system that typically begins in childhood. Batten disease is often fatal by the late teens or twenties.

Building Value and Mutual Benefit through EFPIA's Patient Organisation Code

By Faraz Kermani, Communications Manager (External Affairs) at the European Federation of Pharmaceutical Industries and Associations (EFPIA)

In recent years, there has been a demand towards more transparency in the interactions between patient's groups and the pharmaceutical industry. In this newsletter edition, we asked the European Federation of Pharmaceutical Industries and Associations (EFPIA) to outline the main points of the EFPIA Code on disclosure of transfers of value from pharmaceutical companies to healthcare professionals and healthcare organisations. This is a voluntary code followed by all EFPIA Members. We hope this will provide more clarity on best practices between patients' groups and the pharmaceutical industry.

Over the past decade, the pharmaceutical sector has undergone a significant transition in terms of its drug, regulatory and policy development focus. Patient groups began to give patients a stronger voice and health care stakeholders in the pharmaceutical sector, including industry, regulatory and government bodies, made greater efforts to place the patient at the centre of their work.

This was not a sudden '*conversion on the road to Damascus*', though. Medicines manufacturers had always had the patient in mind, because without patients there simply would be no pharmaceutical industry. It was the value that patient groups brought to the overall drug development and use process that began to gain in significance.

A more unified articulation of patients' needs began to raise awareness that closer collaboration between industry and patient groups would be mutually beneficial. Expertise gained from patient organisations, specifically the recorded experiences of those individuals living with the conditions that manufacturers were seeking to treat, began to shape and guide research and influence the way in which medicines were used. The collaboration between industry and patient groups started to raise disease awareness and saw the launch of a number of patient reported outcomes projects, with the aim of improving treatment pathways and products themselves.

However, co-operation between industry and patient organisations was viewed with some suspicion by some stakeholders. Industry was therefore keen to publicise the fact that this vital relationship is governed by a strict ethical framework.

Co-operation between industry and patient organisations was viewed with some suspicion by some stakeholders. Industry was therefore keen to publicise the fact that this vital relationship is governed by a strict ethical framework.

The European Federation of Pharmaceutical Industries and Associations (EFPIA), which is the representative body of the pharmaceutical industry in Europe, developed and approved a code of practice on relationships between the pharmaceutical industry and patient organisations (hereafter 'PO Code') in 2007. The code does not prejudice national law and its regulations were transposed into each national industry association codes. Moreover, EFPIA association members had to adopt provisions in their national codes, which are at least as rigorous as the PO Code provisions.

Revised in June 2011, the purpose of the code was firstly to recognise formally the many common interests that the pharmaceutical industry shares with patient organisations, which represent and/or support the needs of patients and/or caregivers. Secondly, the code lays down principles for maintaining transparent and ethical relationships with these organisations so that co-operation does not detract from the ultimate aim of serving the patient.

In the interests of transparency, the PO Code requires pharmaceutical companies on a yearly basis to publish a list of patient organisations to which they provide financial/indirect/non-financial support or contracted services payments. This information has to contain a description of the nature and monetary value of the financial support, together with any associated costs. While safeguarding confidential information, it also has to be written in such a manner as to make it accessible to the lay-person. The interaction between pharmaceutical companies and patient organisation should therefore be able to withstand any rigorous public scrutiny.

Another significant provision that seeks to ensure that the patient organisation/industry relationship is ethically sound is the rule that a single company may not demand to be the sole funder of a patient organisation. Guarding against undue influence, this rule stems from competition considerations and therefore underscores the need for patient organisations to diversify their funding sources.

The PO Code recommends a written agreement between both parties that defines both financial and non-financial support. This agreement should include the amount of funding and its proposed use, and/or the purpose and description of any other form of non-financial assistance.

The aim is to guarantee that patient association activities remain fully independent. It prevents pharmaceutical manufacturers, for example, from influencing printed materials published by a patient organisation that they may sponsor, with a view to promoting their own commercial interests.

This approach fully supports the prohibition on the advertising of prescription-only medicines to the general public, which is upheld in full by the PO code, in line with Directive 2001/83/EC of 6 November 2011 on the Community code relating to medicinal products for human use.

The PO Code in fact ensures that patient organisations may not be witness to illicit promotional efforts, including through medical representatives visits or congresses, for example. Therefore, the same openness applied to financial and non-financial aid has been carried through the EFPIA code to apply to events and hospitality provided to patient organisations, which are in fact a reflection of identical rules applied to interactions with healthcare professionals in this area.

The event itself must serve a recognised purpose, such as increasing disease awareness, or it must be in some way justifiably informative or educational. The PO code ensures that any hospitality provided by the pharmaceutical industry to patient organisations and their members is reasonable and linked to the main purpose of the event. Hospitality is limited to travel, meals, accommodation and registration fees.

The EFPIA PO code has already achieved its major task of harmonising the rules across Europe that apply to the relationship between the pharmaceutical industry and patient organisations. It continues to serve as a valuable framework in which to share scientific knowledge and learn from patient expertise and experience. Both sides are committed to a mutually beneficial exchange and this type of relationship can only be carried out in compliance with ethical rules.

The pharmaceutical industry fully recognises the continued importance of the interaction with patient organisations going forward and continues to evolve the PO code to meet new challenges, as they occur. The aim is to ensure that patients can continue to have confidence in the relationship between industry and the patient organisations that represent them. Effective co-operation between both sides can help secure the evolution of innovative medicines for the continued advancement of patient care.

The full EFPIA code can be consulted on the [EFPIA website](#).

Announcements

EHC 2015 Calendar of Events

- Jun 15** Round Table on 'Tenders and Procurement' - open to NMOs and selected participants
Brussels, Belgium
- Jul 3-5** Youth Workshop - open to NMOs only
Rome, Italy
- Sep 11-13** HTA and Economics Workshop - open to NMOs only
St Petersburg, Russian Federation
- Oct 1-2** Pilot Leadership Conference - open to NMOs only
Belgrade, Serbia
- Oct 2-3** EHC Annual Conference - open to all
Belgrade, Serbia
- Oct 4** EHC Annual General Assembly - open to NMOs only
Belgrade, Serbia
- Nov 20-22** New Technologies Workshop - open to NMOs and selected participants
Location to be determined
- Nov 30** Round Table on 'Mild Haemophilia' - open to NMOs and selected participants
Brussels, Belgium

To find out more about EHC events visit <http://www.ehc.eu/calendar-of-events/events/>

Other Events

- May 7-10** World Federation of Hemophilia Musculoskeletal Congress
Belfast, UK – More information at www.wfh.org/whd
- May 19** EPF Annual General Meeting
Brussels, Belgium – More information at <http://bit.ly/1yiAhHy>
- May 20-21** IPFA/PEI 22nd International Workshop on 'Surveillance and Screening of Blood Pathogens'
Prague, Czech Republic - More information at <http://bit.ly/1tnjXfe>
- May 20-21** EPF Conference on Patient Empowerment
Brussels, Belgium – More information at <http://bit.ly/1EqHdWp>
- May 28** 2015 Annual Workshop of the European network of paediatric research at the European Medicines Agency (Enpr-EMA)
London, United Kingdom – More information at <http://bit.ly/1NkiYN4>
- May 29-30** EURORDIS Member Meeting
Madrid, Spain – More information at <http://bit.ly/1yiAs5I>

- June 5** Joint Drug Information Association (DIA)/European Medicines Agency (EMA) Information Day on Post-Authorisation Studies (PAS)
London, United Kingdom – More information at <http://bit.ly/1D2MOgR>
- Jul 2** EPF 2015 Conference on Cross-Border Healthcare: Bringing Together National Contact Points and Patients' Organisations across the EU
Brussels, Belgium – More information at <http://bit.ly/1OiLVFZ>
- Sep 28-29** IPFA /BCA 2nd Global Symposium on 'The Future for Blood and Plasma Donations'
Fort Worth (Dallas), USA – More information at <http://bit.ly/1NZnCec>
- Dec 1-2** IPFA Workshop on Improving Access to Plasma and Plasma Products in the Southern African Region
Stellenbosch (Cape Town), South Africa – More information at <http://bit.ly/1JvK5Pr>

EHC Conference 2015 – Preliminary Programme

2-5 October 2015, Belgrade Serbia

Friday 2 October		
08.00-19.00	Registration; Hospitality Desk	
09.00-19.00	Exhibition & Poster Display	
12.00-13.00	<i>Buffet Lunch</i>	
13.00-13.40	Haemophilia in Serbia Chair: Dr Danijela Mikovic	WORKSHOP 1: To Be Confirmed and OPEN TO ALL Facilitator:
13.00-13.20	Comprehensive care and inhibitor treatment <i>Prof Predrag Miljic</i>	
13.20-13.40	The rarer bleeding disorders in Serbia and Europe <i>Dr Danijela Mikovic</i>	
13.40-14.00	Gene therapy <i>Prof Flora Peyvandi</i>	
14.00-15.00	Women and bleeding disorders Chair: Prof Angelika Batorova	
14.00-14.30	Genetic and bleeding risk in carriers of haemophilia: diagnosis and care <i>Dr Roseline d'Oiron</i>	
14.30-15.00	Pregnancy and menorrhagia <i>Prof Rezan Khadir</i>	
15.00-15.30	<i>Tea/Coffee & Poster Session – Exhibition Area</i>	
15.30-17.00	Complications in Haemophilia Chair: Prof Paul Giangrande	WORKSHOP 2: Women in the bleeding disorder community: Starting a European conversation OPEN TO ALL Facilitator: Yannick Collé Speakers: Christina Burgess, Evelyn Grimberg, Helene Døscher
15.30-16.00	Thrombosis in bleeding disorders <i>Prof Philippe De Moerloose</i>	
16.00-16.30	Ageing and cardio-vascular health <i>Prof Pier Mannucci</i>	
16.30-17.00	Orthopaedic surgery <i>TBC</i>	
17.00-17.30	<i>Tea/Coffee & Poster Session – Exhibition Area</i>	
17.30-19.00	Pharmaceutical Symposium 1 - Sobi	
19.30-21.00	Welcome Reception and Buffet Supper Brian O'Mahony, EHC President Vladimir Ilijn, Hemophilia Society of Serbia TBC Minister of Health or Mayor	

Saturday 3 October		
08.00-19.00	Exhibition; Poster Display; Registration; Hospitality Desk	
08.30-10.00	Pharmaceutical Symposium 2 - Pfizer	
10.00-11.00	Inhibitors Chair: Prof Mike Makris	
10.00-10.30	Management of inhibitors	

	<i>Prof Mike Makris</i>
10.30-11.00	Risk factors <i>Prof Jan Astermark TBC</i>
11.00-11.30	<i>Tea/Coffee & Poster Session – Exhibition Area</i>
11.30-13.00	Pharmaceutical Symposium 3 - Baxter
13.00-14.00	<i>Buffet Lunch – Exhibition Area</i>
14.00-16.00	Long-acting factors Chair: Prof Flora Peyvandi
14.00-14.20	Clinical experience <i>Prof Manuel Carcao</i>
14.20-14.40	Measuring lab assays <i>Prof Jovan Antovic</i>
14.40-15.00	PK-guided treatment <i>Prof Paul Giangrande</i>
15:00-15:20	Post-marketing surveillance and failures <i>Prof Flora Peyvandi</i>
15:20-15:40	Economics <i>Brian O'Mahony</i>
15.40-16.00	Discussion
16.00-16.30	<i>Tea/Coffee & Poster Session – Exhibition Area</i>
16.30-18.00	Pharmaceutical Symposium 4 – Novo Nordisk
19.00-19.30	Coach Departure for City Tour
20.00-24.00	Conference Dinner
Sunday 4 October	
09.00-13.00	Exhibition; Poster Display; Hospitality Desk
09.00-11.00	EHC General Assembly (<i>open to NMOs only</i>)
11.00-11.30	<i>Tea/Coffee – Exhibition Area</i>
11.30-13.00	EHC General Assembly (<i>open to NMOs only</i>)