EHC
european haemophilia consortium

EHC NEWSLETTER

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EHC Newsletter December 2016

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President and CEO report

End-of-year message

We are proud to end 2016 together with all of you. It has been an ambitious, productive and rewarding year. We have worked closely with a wonderful, growing team of volunteers and staff and also had the pleasure to start new collaborations inside and outside of our growing community, including new partners from other disease areas, industry and policymaking.

Brian O'Mahony is the EHC President. Amanda Bok is the EHC CEO.

New networks

As 2016 comes to a close and 2017 is around the corner, we are particularly pleased to announce an ambitious, multi-stakeholder project that was given its wings this autumn when our General Assembly unanimously approved PARTNERS. Standing for 'Procurement of Affordable Replacement Therapies – Network of European Relevant Stakeholders,' this multi-year programme was built to help people with haemophilia and their governments to gain significantly increased access to factor concentrates through a sustainable and step-wise approach. By working closely with key industry partners, national decision-makers, clinicians and patient representatives, we hope to help set the right framework for multi-annual purchasing at affordable prices for national budgets in up to 14 European countries, where FVIII per capita use is less than 4 International Units per capita and not every child has access to prophylaxis. Our work will begin in February 2017, when patient leaders and key clinicians from up to 14 countries will meet with us in Paris.

We are also pleased to announce that this month EU Member States officially approved EuroBloodNet, the European Reference Network (ERN) for rare haematological disorders! Rare bleeding disorders and haemophilia will only be a small part of what EuroBloodNet will cover (and time will tell how well this network will be able to support our small community). But we now raise our proverbial glasses to the mammoth, multi-stakeholder achievement that has been EuroBloodNet's birth and look forward to actively promoting its further development as it unfolds, hopefully with the same meaningful and substantive patient involvement as at its birth.

Partnerships

The EHC is pleased to announce one new and one revised formal partnership. This autumn we signed a Memorandum of Understanding (MOU) with the United Nations Development Program (UNDP) in Ukraine, with whom we will join our efforts towards ensuring best practices in the procurement of coagulation factor concentrates in Ukraine. We also signed an updated MOU with the World Federation of Hemophilia (WFH) and that document is available on our website.

Highlights of the autumn

In addition to the above, we held our first in the new 'Tenders and Procurement' workshop series in Baku, Azerbaijan, in September (see pg 11) with a very active and engaged group of Russian-speaking NMOs. Our EHC Conference in Stavanger (see pg 8) was warmly and successfully hosted by the Norwegian Haemophilia Society, to whom we are grateful, and we are pleased to report that we received glowing evaluations on the programme content and interactivity from participants. We ourselves were also deeply moved to welcome our Dutch cyclists from '*Bloodrelatives*' (see page) who, collectively, travelled more than 10,000 km (1,000 km each) to raise awareness while getting there from Utrecht by bike! And the inspiration didn't stop there. In December we organised Europe's first Inhibitor Summit for people and families with haemophilia and inhibitors (see pg 16) and were both deeply moved by their stories while also left fired up to maximize what we can do for them through the European Inhibitor Network in 2017. Under that program, we also worked closely with key European health care providers towards 'European Principles on Inhibitor Management' and are pleased to announce that they will be finalised and hopefully published in early 2017. Finally, we approached the end of the year with our New Technologies workshop (see pg 13) and the last Round Table of the year, focused on Outcome Measures (see pg 18), both of which were held in November and helped set the scene for the future products and decision-making tools that are around the corner.

The cycling team of the Dutch NMO 'Bloodrelatives' cycled all the way from Utrecht to Stavanger

Elections

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

The EHC Annual General Assembly elected new steering committee members in 2016

its first meeting in December. Finally, we extend a special thank-you to outgoing SC members Traci Marshall-Dowling (Irish NMO) and Michael van der Linde (Dutch NMO) who contributed so much to the EHC and we are grateful that they will both stay involved in our Youth Committee.

Staff and office changes

We are pleased to announce the addition of Raia Mihaylova, who will join us in January as full-time communications officer based in Brussels and we thank Laura Savini, who has done an excellent job in her joint role as communications and public policy officer since joining the EHC in 2014. Laura will continue on with the EHC as full-time public policy officer. In mid-January the EHC will also move offices, taking a larger space in the same building to better accommodate our growing staff team as well as host volunteers and visitors – feel free to come by and see us!

Raia Mihaylova will join the EHC team in 2017 as EHC Communications Officer

Looking ahead to 2017

Last but not least, we close 2016 with two planted seeds. First, we endorsed the efforts of a very dedicated group of women in Europe and will now have an official EHC's women's group, whose work and membership we look forward to announcing in greater detail in our next newsletter. Second, we launched our Europe-wide survey on haemophilia and hepatitis C, which we hope will continue to inform and help us fine-tune our advocacy work in this area in 2017. Finally, we set and circulated our 2017 event dates, which you can find in your emails, on our website and on the last page of this newsletter. We look forward to seeing all of you again in 2017 and in the meantime extend our heart-felt wishes to all of you for very:

Happy holidays!

EHC News

Comprehensive care in haemophilia: Hepatology

Prof Geoffrey Dusheiko interviewed by Laura Savini***

'Hepatitis C (HCV) eradication is within easy reach for people with bleeding disorders in Europe in the short-term future.' This was the message continuously spotlighted [1] by the European Haemophilia Consortium (EHC) in 2016.

In fact, the advent of effective, easy-to-take and pangenotypic treatments (i.e. direct acting antivirals or DAAs) in Europe gives policy-makers and healthcare professionals tools to make HCV eradication within the bleeding disorder community a reality. This should be done to right the wrongs that occurred during the contamination tragedies of the 1970s through to the early 1990s.

For the final issue of our comprehensive care and haemophilia spotlight of the year, we interviewed Prof Geoffrey Dusheiko, an emeritus professor of medicine at the Royal Free Hospital (London) and the University College School of Medicine (London). Prof Dusheiko's research interests include the management and treatment of hepatitis B and C and small hepatocellular carcinoma. He has also a special interest in research in viral hepatitis, focused on viral genotyping, applied molecular virology, the natural history of chronic viral hepatitis and antiviral therapies. Prof Dusheiko has been supporting the haemophilia community with his expertise on the liver and liver diseases for many years. We are delighted to hear his perspective on how hepatology fits into the comprehensive care of people with haemophilia and other bleeding disorders.

On hepatology

Hepatology is a medical discipline related to the study of the liver. It is part of the larger medical discipline of gastroenterology, which refers to the study of the bowels and the gut. Hepatology also refers to the study of hepatobiliary pancreatic diseases. It is both a medical and a surgical discipline.

On hepatology and haemophilia

In the 1970s and through the early 1990s the quasi-totality of the haemophilia population globally was infected with viral hepatitis C (HCV). Many of the surviving patients are still struggling today with HCV and the co-morbidities generated by the disease, such as liver cancer. So it's clearly relevant for people with bleeding disorders who are suffering from HCV to have access to specialist care to manage their liver and liver diseases.

On the importance of treating HCV

HCV is associated with progressive fibrosis, i.e. the scarring of the liver, which prevents the liver from performing its normal functions[2]. Over time fibrosis can lead to cirrhosis, which is frequently irreversible. In turn cirrhosis will lead to the decompensation of the liver causing a series of

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[1] In 2016 the EHC held several events to stress the importance of access to HCV treatment for people with bleeding disorders. In April, the EHC held an event to mark World Haemophilia Day. In June, the EHC organised a Round Table of Stakeholders at the European Parliament. During its Annual Conference, the EHC held a symposium on the topic. Finally, the EHC launched in December its Annual Survey on access to HCV treatment for people with haemophilia in Europe.

[2] The liver has many functions including, for example, regulating the composition of blood, removing toxins, processing nutrients and metabolising alcohol and drugs. Incidentally the liver is also where coagulation factors are produced. People with haemophilia who receive a liver transplant are immediately cured of haemophilia, although this is not a sustainable treatment.

complications including portal hypertension, oesophageal varices, swollen veins in the oesophagus, encephalopathy (i.e. the liver inability to synthesise toxic substances that are normally removed by the liver), ascites (i.e. an accumulation of fluids in the abdomen), edema (i.e. an accumulation of fluids in the body) and recurrent infections. In short, it is essential to avoid cirrhosis because it leads to a number of devastating complications. It is important to be conscious that although cirrhosis may take a long-time to develop (it is estimated that cirrhosis will often develop 20 to 30 years after HCV contamination), it onsets a rapid deterioration of the patient's health. HCV progression can be accelerated by a number of factors including excess alcohol but also the co-infection with the human immunodeficiency virus (HIV).

On the importance of correct genotyping

HCV has six different genotypes and each genotype holds many subgroups. Therefore it is important to identify the correct genotype affecting an individual so that treatment can be tailored to the genotype. It is possible for individuals to be infected with more than one genotype. This is particularly common for people who have been infected through blood and blood product contamination, such as people with bleeding disorders. In this case, treatment should be pangenotypic because if only the dominant genotype is treated the minor genotypes will become dominant after treatment. However the advent of pangenotypic therapies may eliminate the need for genotyping.

Prof Dusheiko gave a presentation during the last EHC Annual Conference

On DAAs

HCV is a RNA virus, however, unlike HIV or hepatitis B the virus does not integrate directly into the host. DAAs directly target the enzymes of the virus (and not the host), inhibiting the virus and thus making the treatment very effective. DAAs are so revolutionary because compared to older generation treatment, i.e. ribavirin and interferon, their response rate is very high and they can be used in patients with both cirrhosis as well as decompensated cirrhosis. Furthermore, their mode of administration is easy, as it is given by mouth, so that even non-liver specialists such as haematologists can administer the treatment by following treatment guidelines. Another positive aspect of DAAs is that the entire treatment course only lasts 12 weeks, which is considerably shorter than previous treatments with interferon and ribavirin that could sometimes last for years. Finally, the response rate in patients co-infected with HIV is exactly the same as in non-co-infected patients. On this note, although the treatment is manageable for patients taking anti-retroviral medicines, caution should be exercised. Clinicians should ensure that there is no drug-to-drug interaction. This is why it is advisable for people with haemophilia who are co-infected with both HIV and HCV to have their haematologist, HIV specialist and hepatologist working together to manage their treatment.

On preserving liver health

It is important that patients affected by HCV look after their liver to preserve its health as long as possible. This can be done by avoiding excess alcohol consumption, which can greatly accelerate

fibrosis. Furthermore, exercising, avoiding obesity and avoiding smoking can all support liver health.

On the work of hepatologists

The work of a hepatologist is very diversified. In his position Prof Dusheiko fulfils educational, research and clinical roles. For instance, Prof Dusheiko advises policy-makers from various countries on policies to tackle HCV prevention and treatment. He also advises pharmaceutical companies on the development of new compounds. Finally he performs liver clinic duties, looking after patients affected by viral hepatitis.

On treating PWH with HCV

In the past the main concern for treating people with bleeding disorders was the risk of bleeds that could have been caused by liver biopsies. These were fairly invasive and required the need for coagulation factors. Alternatively, transjugular liver biopsy could have been performed on these patients as they are less invasive. However, the need for liver biopsies has now much diminished thanks to non-invasive procedures to assess the stiffness of the liver, which provides information on the progress of the liver disease. These tests can now be done with technologies such as transient elastography (i.e. Fibroscan) or even simple blood tests. When patients need other invasive procedures such as endoscopy there needs to be caution of the risk of bleeding, although this risk is low. The same caution should be applied when doing procedures like paracentesis (i.e. the removal of excess body fluids), for example, for patients with ascites. Although this procedure can also be easily managed.

"HCV is eminently treatable and [...] the aim should be to treat all patients to preserve their liver health, avoid complications and eradicate HCV," states Prof Dusheiko

On working with other healthcare professionals

Close collaboration is necessary amongst various healthcare professionals such as haematologists, hepatologists and HIV specialists to manage patients with bleeding disorders and co-infections. This is to gain better understanding on the overall health status of the patient but also to ensure that drug interactions are compatible.

On hepatology training

There is no standard training for hepatologists in Europe and the situation is vary variable. Many hepatologists are trained as gastroenterologists with a special interest in hepatology. However, there are many excellent professional organisations focusing on the study of the liver such as the European Association for the Study of the Liver (EASL) in Europe and many similar organisations in other world regions. These organisations offer annual scientific conferences as well as many professional trainings. Furthermore they produce treatment guidelines. Finally, these networks foster knowledge exchange and the development of an international scientific community.

On current and upcoming challenges in hepatology

HCV is eminently treatable and the primary barrier to eradicating the disease is the financial impact of these medicines on healthcare systems. This is due to the large number of patients to be treated. As a result, in many countries access to treatment is restricted to those patients in the most advanced stages of HCV. However the aim should be to treat all patients to preserve their

liver health, avoid complications and eradicate HCV. In this regard it is important to convince policy-makers to identify HCV reservoirs and to educate the general population about HCV mode of transmission and risky behaviours. This is to avoid new infections and to ensure that there is no re-infection in treated patients. With regard to the blood supply, it has generally become safer thanks to better donor screenings and viral inactivation or removal techniques. However concerns remain in some parts of the world.

At the moment there are excellent generic compounds that have been developed thanks to technology transfers. This has led some patients who are unable to receive treatment in their country to purchase these generic treatments online. Although this situation leads to complex legal and ethical issues and discussions, in practical terms, Prof Dusheiko would urge patients who decide to purchase generic medicines online to exercise extreme caution. Patients should ensure, as much as possible, that they purchase actual medicinal products and not counterfeit medicines that could further harm them.

**Prof Geoffrey Dusheiko is emeritus professor at the University College London.*

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

EHC Annual Conference: The European bleeding disorders community meets in Norway

By Kit Greenop and Yordan Aleksandrov, consultants at RPP Group

Each year the European Haemophilia Consortium (EHC) organises its Annual Conference to bring together the European bleeding disorders community to discuss the latest scientific and policy developments. In 2016, the 29th Conference was held in Stavanger, Norway in cooperation with the EHC Norwegian National Member Organisation (NMO), Foreningen for blødere i Norge.

The three-day event was attended by more than 300 participants, while panel discussions focused on the crucial topics for the rare bleeding disorders community, such as treatment availability, gene therapy and disparities in access to haemophilia treatment and care within and between the European countries.

The EHC wishes to thank the volunteers from the Norwegian NMO for their help during the conference

The sessions of the event focused on the following topics:

- Organisation of haemophilia care in Norway and other Nordic countries
- New developments in Haemophilia care
- Inhibitors

- Clinical and youth debates
- Hepatitis C
- Patient-reported outcome data

Multiple symposia were organised, aimed at exchanging best practices between NMOs, academics and industry. In addition, screening of the documentary "Haemophilia Stories" from the director Goran Kapetanovic was also organised. The movie was created in 2015 and depicts the daily lives of people with haemophilia in five European countries. The movie shares the stories of the individuals of different generations and with different types of haemophilia, living across Europe. The documentary is currently available on the EHC webpage, please click here to access it.

Main highlights

Perspectives from the Nordic countries were presented by Prof Pål André Holme from Oslo University Hospital (Norway) and Prof Jan Astermark from Lund University (Sweden). The lively discussions provided participants with the present situation of haemophilia treatment in Sweden and Norway. Currently in Norway, home treatment is widely available and the country, despite its geography, has managed to create a system with only one specialised centre in Oslo. Prof Holme argued that fewer centres in Europe would be a more suitable approach for haemophilia patients. This is certainly a topic for debate in future discussions as the prevalence of centres of expertise in Europe grows.

Prof Pål André Holme gave an overview of haemophilia care in Norway

During the conference, new developments in haemophilia care such as gene therapies were also discussed. These topics bring with them great excitement and anticipation and these presentations aimed to deliver messages on the stage of development of new products and expected advantages and challenges. The financial challenges of the therapies were emphasised by Prof Edward Tuddenham from the University College London, Prof Johannes Oldenburg from the University Clinic in Bonn and Prof Cedric Hermans from Saint-Luc University Hospital in

Young volunteers challenged established clinicians during the youth-clinician debates

Brussels. Patients noted, however, the crucial hope that new therapies bring for a stigma-free life, a somewhat intangible concept, which was revisited when discussing outcome measures.

The most engaging part of the conference were the vibrant youth debates (see page 52) between academics and patients, including volunteers from the Netherlands who participated in an

awareness campaign by cycling some 1,000 kilometres from Utrecht to Stavanger (see page 29) and arriving on the morning of 8th October. The clinicians-youth debates addressed the main challenges and issues in haemophilia and concluded with a note that there is a clear need of prophylaxis for adults; significant time should be spent on inhibitor patients, and access to hepatitis C (HCV) treatment should be a priority for NMOs. A second set of stimulating interventions produced three draws in the lively clinician-against-clinician debates on gene therapy, extended half-life factors and caesarean for carriers.

Furthermore, panellists presented the success stories of Portugal and Ireland in HCV treatment and helped spread inspiring stories about how to ensure that governments appropriately deal with these afflicted patients. In Portugal, there is a current success rate of 96 per cent of HCV treatment in the haemophilia community, while in Ireland every haemophilia patient with HCV has either had a treatment or is currently on treatment. For better future cooperation, it was proposed to connect the health data from the haemophilia centres, as in some countries (e.g. the United Kingdom), there is a clear number of people affected by HCV but no indication of how many of them are/were treated. On this note the EHC decided to launch in 2016 an annual survey amongst its members to identify the number of people in the bleeding disorder community affected by HCV (see page 5).

Helene Døscher (left hand-side) is the Chairperson of the EHC Norwegian NMO

The Annual Conference was also accompanied by industry symposia and discussions, which focused on partnerships within the clinical trial landscape, improving global haemophilia care, patient reported outcomes and better access to haemophilia care. Inhibitors and the need for physical activity in haemophilia were also discussed and presented by the '*Bloodrelatives*' cyclists who cycled from Utrecht to Stavanger, as an awareness-raising activity developed by the Dutch NMO. The conference was held in English with simultaneous Russian interpretation.

The EHC would like to warmly thank the Foreningen for blødere i Norge for hosting this conference and all volunteers who contributed to the success of this event. The next EHC Conference will be held from 6-8 October 2017 in Vilnius, Lithuania. More information about the programme and registrations will be available in the new year on the www.ehcconference.org website.

EHC holds first workshop on Tenders and Procurement

By Laura Savini, EHC Communications and Public Policy Officer

In September the European Haemophilia Consortium (EHC) held its first workshop on Tenders and Procurement in the beautiful city of Baku, Azerbaijan, also known as the Paris of the East for its European-inspired architecture. This was the first of a new series of three yearly workshops.

Participants during the EHC workshop on Tenders and Procurement

The EHC believes that patients with bleeding disorders, which are rare, congenital and chronic conditions, are expert in their disease area. As such, they should be actively involved in all aspects of the organisation of haemophilia care in their own countries. Tenders and procurements, i.e. the purchasing of medicinal products, to treat haemophilia and other rare bleeding disorders are an important process for patients. In fact, the quality and quantity of medicinal products purchased in a given country relies on how the purchasing processes are carried out. As a result patients' access to treatment will depend on the outcomes of these tenders and procurements, which could be either excellent - meaning adequate access to high quality medicinal products – or poor – meaning low access to medicinal products of dubious quality and safety profiles. The EHC believes that these purchase processes should be carefully planned ahead to allow the greatest number of pharmaceutical manufacturers to be able to take part in them. Furthermore, they should be carried out in a transparent manner and involve all stakeholders including patient representatives, healthcare professionals, representatives from insurance companies, health ministries and other relevant government agencies. On this note the EHC showed through its 2014 annual survey that countries actively and meaningfully involving patients and healthcare professionals in their tender and procurement process were more likely to provide adequate and cost-effective treatment to their patient population.

The problem that is encountered in most European countries is that coagulation factor concentrates are purchased like any other hospital or medical goods. This means that government representatives, who are experts in the technicalities of the purchasing process, are assigned with the task of carrying out the purchase. Unfortunately, most of the time they do not know much about the specificities of haemophilia and other rare bleeding disorders. For example, government experts may just assume that the products are safe but patient representatives may

ask questions pertaining to safety measures in the manufacturing process such as blood donor selection criteria and viral inactivation or removal. Although our readers from within the European Union (EU) may take these safety measures for granted, this is not always the reality in countries with less strict regulations on blood safety. Hence the importance of having patients around the table who will not accept anything less than 100 per cent safety and quality for their own community.

It is on these premises that 17 participants from Azerbaijan, Bulgaria, Georgia, Kyrgyzstan, Latvia, Montenegro and Ukraine embarked on an intensive two-day training. These participants included patient representatives and healthcare professionals but also many government officials involved in the procurement of coagulation factor concentrates in their country. The EHC really wishes to stress its gratitude to these officials for showing their willingness to learn more about haemophilia and to actively engage with patients. We believe that this is an incredibly promising step paving future collaboration and ensuring that patients in their respective countries receive safe and efficacious treatment. The training was provided in English and Russian with live-interpretation (see pg 22).

The first day of the workshop was dedicated to theoretical learning. Participants learned more about the history of haemophilia treatment, from cryoprecipitate to gene therapy; blood safety covering the lessons learned from the contamination tragedy but also regarding the role of patient organisations in advocating for safer blood supply; as well as tenders and procurement models. Mr Glenn Pierce, a long-standing member of the haemophilia community and board member of the World Federation of Hemophilia (WFH), Mr Yurii Zuylov, President of Всероссийского общества гемофилии, the EHC Russian National Member Organisation (NMO), Mr Brian O'Mahony, EHC President, and Mr Virinder Sethi, Senior Regional Brand Manager from Pfizer presented on these topics.

On the second day, delegates took part in a mock tender board meeting

The second day was devoted to practical exercises in which participants were asked to reproduce the work of a tender board including developing and filling in scoresheets for the appraisal of potential medicines seeking marketing and reimbursement in a given country. This day was highly interactive and generated many relevant questions and discussions with regard to the selection process of coagulation factors concentrates.

This was the second workshop, specifically organised for EHC's Russian speaking members (i.e. NMOs from countries where Russian is the vehicular language instead of English). Participants were engaged and the general atmosphere and feedback received was very positive. Participants asked for further training in the area but also noted that they were going to disseminate the lessons learned within their country upon return.

This workshop was made possible thanks to an educational grant from Pfizer, whom the EHC wishes to thank for its support. *The next edition of the EHC Workshop on Tenders and Procurement will take place on 15-17 September 2017. Registrations for this workshop will open on 12th June.*

A Review of the EHC Workshop on New Technologies in Haemophilia Care

By Seamus McDonald and Laura Savini***

For the third consecutive year, representatives from the European Haemophilia Consortium (EHC), health care professionals and regulators reconvened in November to discuss novel and upcoming medical technologies in haemophilia treatment. Seamus McDonald and Laura Savini report.

This year, the event was held in Berlin and coincided with the last European tour of US President Barack Obama who was in the German capital the week of the event. While President Obama warned European allies about '*an impending shift in the global order that could lead to a meaner, harsher, more troubled world*,' Brian O'Mahony, EHC President, opened the event with a similar albeit more positive message for the haemophilia community. We are in fact about to see (or so it is hoped) an impending shift for treatment options for people with bleeding disorders. However, in the case of the haemophilia community, these could bring beneficial changes ranging from longer-lasting treatment, higher trough levels and better treatments for people with inhibitors. However, participants were warned that although the future is promising, as with every new treatment, scientific and medical development, caution needs to be exercised. So together with other 50 participants from 15 European countries and colleagues from Canada, we set ourselves to learn about what new technologies are currently being developed or marketed in the area of haemophilia.

Participants during the EHC Workshop on New Technologies in Haemophilia Care

To read about all of the new medicinal products being developed in the area of haemophilia, we advise you to go to the EHC September 2016 Newsletter and read the comprehensive article by Glenn Pierce (it's nine pages long! so make yourself a cup of tea and settle in for an exciting read). However, we will tell you that the workshop covered important topics such as immunogenicity of coagulation factor VIII, inhibitor tolerance induction, advances in gene therapy and extended half-life (EHL) products. For all of these we discussed what added value these treatments could bring to patients in terms of improvement of quality of life. However we also noted that long-term safety is unclear (as these products are new) and although immunogenicity data is good for the clinical studies, we have yet to see how these products will '*behave*' in the real world.

In our personal opinion the workshop was a great success, the talks were well organised and the quality of speakers was exceptional. We also believe that the need for a workshop such as this is evident following the influx of new products and treatments. It is very enriching to be able to discuss with peers from all over Europe about these new products and what they could concretely mean for patients in various European countries. Besides the technical and scientific knowledge acquired during the workshop, it is also always exciting to meet old friends and new and to be able to take the temperature on what people and EHC National Member Organisations (NMOs) think.

The workshop kicked off with a talk from Dr Mirella Ezban, Scientific Vice-President Coagulation Factors at Novo Nordisk on laboratory assays for EHL products. Although the topic is more technical, it is nonetheless very important and, if not properly addressed, may cause much harm to patients. It was therefore quite interesting to learn how Dr Ezban and her team tried and tested many assays to ensure that coagulation factors were correctly measured.

The experience was very enriching and we strongly encourage other participants to attend these meetings to further their knowledge, to ask questions and share experiences with colleagues from across Europe.

Prof Flora Peyvandi, Director of the Angelo Bianchi Bonomi Haemophilia and Thrombosis Centre in Milan and member of the EHC Medical Advisory Group gave an in-depth overview of the newly licensed and soon to be licensed EHL products. Her talk also clearly explained the possible benefits EHL products could offer for haemophilia treatment including fewer infusions, preservation of venous access, and higher trough levels that could lead to less joint damage by reducing or even eliminating microbleeds, thereby also allowing for more active lifestyles.

Saturday morning also saw a strong discussion on the immunogenicity of coagulation factor concentrates both in previously untreated patients (PUPs) and previously treated patients (PTPs). Dr Andreas Tiede from the Medical School in Hannover gave a somewhat controversial talk on the immunogenicity of current FVIII replacement products in PUPs. Naturally, the findings of the Survey of Inhibitors in Plasma-Products Exposed Toddlers (SIPPET) were discussed and we heard experiences from two German physicians in the room: one only treating PUPs with plasma-derived coagulation factor concentrates and the other only treating PUPs with recombinant products. Both had a track record of almost zero per cent of inhibitor development in their PUPs. This led to questions on whether other factors (besides the type of coagulation factor concentrate, i.e. plasmatic or recombinant) used could lead to higher inhibitors such as for example the dosage and the treatment administration mode. The SIPPET study has generated much controversy in the clinical and scientific community who are still a long way from agreeing on how the study results should be translated into clinical practices. Prof Frits Rosendaal from Leiden University gave a much less controversial talk on immunogenicity in PTPs, which also instigated exciting discussions.

Prof Ted Tuddenham from University College London spoke of the advances in gene therapy for both haemophilia A and B. His talk covered multiple gene therapy trials, the issues that they have faced and the issues they may still face in the future as well as possible methods of overcoming these obstacles so that they can reach their goal of a constant and suitable level of factor

expression in every patient. Prof Tuddenham also touched on other genetic methods such as gene editing.

Seamus McDonald is a volunteer with the Irish Haemophilia Society

The day continued with talks on biosimilars, given by Dr Annaliese Hilger, Chair of the Blood Products Working Party (BPWP) at the European Medicines Agency (EMA). Then Prof Peyvandi gave a presentation on inhibitor treatment exploring current and potentially upcoming medicinal products. The talk was exciting, although for those concerned by inhibitors, we would strongly recommend to follow the work of the EHC European Inhibitor Network (see page 16). Dr Giuseppe Mazza, EHC Steering Committee member and a research fellow at the University College London, gave us an insight into the potential of cell therapy for haemophilia. The area is still very much in the experimental phase although it could develop some exciting therapies. The last presentation from the day was by Mrs Glenda Silvester, former secretary for the EMA BPWP; who gave an overview about European regulations on clinical trials and how these serve patients in Europe. This was followed by discussions about clinical trials but also about topics reviewed during the day.

The second day of the workshop was devoted to the appraisal of haemophilia products and patients' involvement in this appraisal process. We heard from Dr Karin Berger from the University of Munich, Ms Sara Schlenkrich from Deutsche Hämophilie Gesellschaft, the EHC German NMO, and Mr Jamie O'Hara, Chair of the EHC Data and Economic Committee. Finally participants were presented with the recommendations from the fourth Wildbad Kreuth initiative, developed from the European Directorate for the Quality of Medicines and Healthcare (EDQM – part of the Council of Europe), the University of Munich and the Rudolf-Marx-Foundation. This initiative looks at best practices for treatment of haemophilia in Europe. The last meeting held in May 2016 resulted in a series of recommendations on various aspects of haemophilia care, which will be presented at the 2017 World Hemophilia Day event organised in Strasbourg on 19th April (see page 57).

In short, the experience was very enriching and we strongly encourage other participants to attend these meetings to further their knowledge, to ask questions and share experiences with colleagues from across Europe.

The EHC Workshop on New Technologies in Haemophilia Care was made possible thanks to educational grants from Roche, Shire and Sobi. The EHC wishes to thank these companies for their support in educating patients across Europe. The 2017 New Technology Workshop is planned to take place from 17th to 19th of November. Registrations will open on 7th August.

**Seamus Mc Donald is a volunteer with the Irish NMO*

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

The first step towards a living network of people with inhibitors in Europe

By Kristine Jansone, EHC Inhibitor Programme Officer

In December 2016 the European Haemophilia Consortium (EHC) held its first Inhibitor Summit, an event bringing together people affected by bleeding disorders and inhibitors from across Europe. The event is part of the EHC's ambitious European Inhibitor Network (EIN), which seeks to develop a network for people affected by inhibitors, reduce social isolation and improve standards of treatment and care. Kristine Jansone, EHC Inhibitor Programme Officer reports on the first edition of the Inhibitor Summit.

"*The best session for me was the peer-to-peer session for parents. I wish it would have lasted longer, I have so many questions!*" wrote one of the participants of the EHC Inhibitor Summit.

From the perspective of the EHC, this was the most important outcome of the EHC Inhibitor Summit as one of its goals was to establish a living network of people with inhibitors, their parents and caregivers from across Europe. Building a community of this group of patients on a European level is an essential element of the EHC EIN programme, as the number of patients nationally is too small to allow NMOs to develop specific programmes and to provide meaningful support to the people affected by a bleeding disorder and an inhibitor.

The event took place from 1st to 4th of December in Barretstown, Ireland, and brought together more than 100 participants, speakers and facilitators from 26 European countries and beyond (we had observers and moderators from the United States and Canada!). The participants were of all ages including children with inhibitors, their siblings and parents, as well as adults with inhibitors and their caregivers and partners. All of these participants brought along many different experiences and good practices that they were eager to share and learn from.

In addition, a variety of expert inputs were offered to the participants, including from scientific, medical and practical perspective.

Prof Paul Giangrande, Chair of the EHC Medical Advisory Group, spoke about inhibitors and their causes, as well as about prophylaxis and possibilities of surgery in patients with inhibitors. His presentations were complemented by Dr Beatrice Nolan who spoke about the new treatments for people with inhibitors.

On a more practical side, the physiotherapists Paul McLaughlin and Nicola Hubert gave insight into the physiotherapy for adults and children. Dr Axel Seuser together with Mrs Petra Buckova spoke about pain management from the orthopaedic and psychological perspectives.

Each session was followed by discussions and our experts addressed new aspects of the theme they had presented on to provide participants with additional insights.

Furthermore, during the Summit the EHC presented other components of the EIN programme. One of these is the development of standards for the management of patients with bleeding disorders and inhibitors in Europe, which is a collaborative project the EHC is working on with the European Association for Haemophilia and Allied Disorders (EAHAD). We are hoping that such guidelines will help to bridge the gap regarding inhibitor treatment across Europe.

The second initiative presented was the EHC online platform for people with inhibitors, their families and caregivers, which is in development. On this platform visitors will be able to learn about inhibitors and the latest treatment developments as well as to discuss and exchange best practices and tips for unburdening in everyday life.

The participants of the first EHC Inhibitor Summit

The EHC wishes to give its heartfelt thanks to all participants, speakers, moderators and volunteers who made this event possible. We also wish to thank the staff and volunteers from Barretstown for their outstanding support. Last but not least, this event could not have been possible without the support of Shire whom we wish to thank for enabling people with bleeding disorders and inhibitors in Europe to meet, learn and share their experiences. The next EHC Inhibitor Summit will be held from 30th November to 3rd December 2017. Registrations for this event will open on 15th August 2017.

Round-up of EHC Round Tables of Stakeholders of 2016

By Laura Savini, EHC Communications and Public Policy Officer

It has been an exciting and fruitful year for the European Haemophilia Consortium (EHC) in generating discussions on 'hot' topics for the haemophilia community in Europe through its Round Table of Stakeholders. In this article we look at the topics covered this year and what will come in the new year.

The year's events kicked off in February with a Round Table of Stakeholders on Inhibitors in Haemophilia A: a topic generating great controversy. Inhibitor formation can be devastating on the patients' physical and mental wellbeing and straining for caregivers and families. For years scientists have been trying to determine the causes of this treatment's side effect, unsuccessfully. Although the first randomised clinical trial in haemophilia researching the effects plasma-derived versus recombinant coagulation factors concentrates on inhibitor formation has been published, it seems to have only further stirred debates and discussions on the topic. The interest generated by the topic was demonstrated by the event's attendance, which was the highest ever with over 70 participants joining in for discussions.

The second Round Table was held in April at the European Parliament in Brussels and supported by Members of the European Parliament (MEPs) Mr Heinz Becker (Austria/ EPP) and Mrs Sirpa Pietikäinen (Finland/ EPP). The topic of the Round Table was '*Ageing and Haemophilia*' and discussed an unprecedented phenomenon: the life expectancy of people with bleeding disorders is now comparable to that of the general population. This means that healthcare professionals are now confronted to patients never seen before. People with haemophilia who are confronted to age-related co-morbidities raise complex medical questions. On the other hand patient organisations also need to develop new programmes to support this community. Although unique to haemophilia, many of these topics resonated with Mr Heinz Becker who is also the President of the Austrian Alliance for Old People.

Mr Heinz Becker (centre) chaired the EHC Round Table on Ageing and Haemophilia

Mrs Sirpa Pietikäinen supported the EHC Round Table on Ageing and Haemophilia

The third Round Table of Stakeholders looked at the access for hepatitis C (HCV) treatment for people with bleeding disorders. In the 1970s and throughout the early 1990s almost the totality of the haemophilia patient population globally was contaminated with HCV. The EHC believes that HCV can be easily eradicated in the small patient population of people with bleeding disorders while righting the wrongs caused by this public health tragedy. The EHC was fortunate to have MEPs Dr Cristian Busoi (Romania/ EPP) and Dr Andrey Kovatchev (Bulgaria/ EPP) chairing the event. In fact both MEPs are members of the MEP Interest Group for Access to HCV treatment. The discussions were engaging and in the end it was concluded that European

Mr Andrey Kovatchev (left hand-side) and Mr Cristian Bușoi (right hand-side) are members of the MEP Interest Group on Access to HCV treatment

countries need to implement strategies to ensure eradication of the disease. The event was held in June in the European Parliament in Brussels and attended by over 50 participants.

The last Round Table of the year was held on 28th November in the European Parliament in Brussels and gathered over 50 participants. The event was kindly supported by MEPs Mrs Norica Nicolai (Romania/ ALDE) and Mrs Viorica Dăncilă (Romania/ S&D). The event covered the topic of outcome measures in haemophilia. Representatives from patient organisations, healthcare professionals and industry representatives gathered to discuss a topic particularly relevant in view of the development of many novel technologies for the treatment of haemophilia. The discussions concluded that outcome measures should help determine the real impact of novel technologies on the quality of life of patients. Mrs Nicolai, Chair of the event, stressed that much remains to be done to bring adequate treatment to patients in some European regions and in particular in her home country, Romania.

The EHC wishes to thank the all speakers, moderators and participants of the EHC Round Tables for their participation and their help in generating fruitful and thought-provoking discussions. Furthermore, the EHC wishes to express its gratitude to all MEPs supporting the work of the EHC and ultimately supporting the European bleeding disorder community. These include not only those that worked with us in 2016 but also many others who collaborated in previous years such as Dr Paul Rübig (Austria/ EPP), Dr Miroslav Mikolasik (Slovakia/ EPP), Mrs Nessa Childers (Ireland/ S&D) and Mrs Mady Delvaux-Stehres (Luxembourg/ S&D). We are fortunate to have, over the years, had so many supporters who were willing to dedicate their time and energy to our cause. We sincerely hope that we will be able to further this collaboration in the future.

Mrs Norica Nicolai chaired the final EHC Round Table of Stakeholders of the year

We are also pleased to announce that in 2017 we will be holding three Round Table of Stakeholders on: Clinical Trials in Haemophilia (7th March), Orthopaedic Aspects in Haemophilia Care (27th June) and Extended Half-life Coagulation Factors Usage and Measurements (28th November). For any additional information, we advise you to consult our website http://www.ehc.eu/events-category/round-tables/

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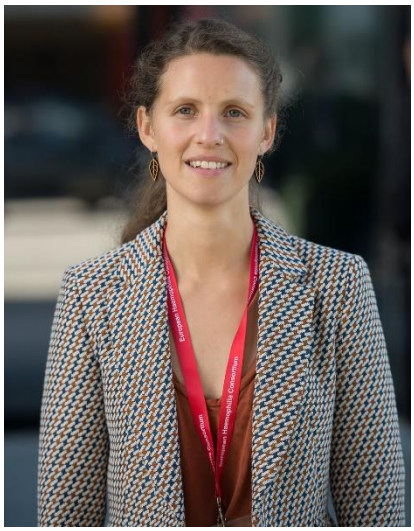
Meet the newly elected EHC Steering Committee

Naja Skouw-Rasmussen and Miguel Crato** interviewed by Laura Savini*** and Olivia Romero-Lux*****

During the 2016 Annual General Assembly of the European Haemophilia Consortium (EHC) two new lay members of the EHC Steering Committee were elected to serve for a three-year term. In this article we meet Ms Naja Skouw-Rasmussen from the EHC Danish National Member Organisation (NMO) and Mr Miguel Crato from the EHC Portuguese NMO.

About Naja



Naja joined the EHC Steering Committe in October 2016

I am 29 years old and have von Willebrand Disease type 2. I am currently working for the National Council for Volunteering (Denmark). I have a background in geography and working in socio-economic, political and environmental issues in Africa, Latin America and Asia.

Since 2008, I have been a board member of Danmarks Bløderforening, the EHC Danish NMO, and as such I have been involved in the strategic planning and logistical organisation of many of the NMOs activities. This has also allowed me to learn more about the haemophilia world but also to provide a new perspective on the way we, as a community, traditionally view and talk about certain topics.

For the past couple of years, my work as a board member has focused on the Danish telemedicine project, which I have been a part of since its inception. This has been an exciting project because it has brought us to collaborate with many different parties, such as healthcare professionals and it has also attracted much political attention in the country. This project has real potential to improve haemophilia care in Denmark three-fold: first by giving patients a tool to track their treatment and in doing so we are hoping that patients will feel more empowered when talking about their medical situation with their physician; second, this project is paving the way for establishing a national registry, which would help us in getting a better sense of the current situation for haemophilia and other rare bleeding disorders in Denmark; and finally, we are hoping that all of this information will contribute to research, establishing better treatment protocols and support the allocation of adequate resources where needed.

I joined the EHC because I believe I can bring useful experiences and fresh energy. First of all I have been sitting on a NMO board for many years and therefore I am quite familiar with working in a team to develop new strategies and activities for an organisation. I am also very familiar with the experience of dealing with healthcare professionals and healthcare systems that have no information about your condition. I believe this is a common situation for many of the EHC members out there and I want to be able to help them in overcoming this challenge. Finally, I want to represent women with bleeding disorders and carriers and promote activities for this patient group. Naturally, I also would like to get involved in existing programmes to continue their successful implementation.

To conclude, I was asked to share something about myself that may surprise people who do not know me and I guess one thing that you may not know is that I have lived in four different continents including North America (Canada), Europe, Africa (Tanzania) and Oceania (Australia).

I am very excited about starting this new adventure and look forward to meeting all EHC members in the coming three years. Finally, I would like to encourage my '*liaison countries*' (i.e. Denmark, Finland, Iceland, Ireland, Israel, the Netherlands, Norway, Sweden and the United Kingdom) to stay in touch and let me know how their national activities are going and if they wish to share something with the EHC Steering Committee or need any support.

About Miguel

My name is Miguel Crato, I am 47 years old and I live near Lisbon with my wife and three sons. I have severe haemophilia A and inhibitors.

Miguel is the President of the Portuguese NMO and a member of the EHC Steering Committee

Since 2013, I have been the president of Associação Portuguesa de Hemofilia, the EHC Portuguese NMO. Throughout my time as a volunteer and since becoming the President of the Portuguese NMO, my biggest priority has been to empower and help members of our community to achieve self-determination and to approach their condition in the most positive way possible. I have tried to achieve this by creating new programmes and events for our NMO members. I am hoping to share this experience with other EHC Steering Committee members and the European community in general. I also really wish to contribute to a harmonisation for better treatment and care of haemophilia and other bleeding disorders in Europe.

To conclude the interview, I was asked to share something about myself that may surprise readers. Although I live in a warmer climate, I really enjoy the cold weather!

As Naja, I would also encourage my '*liaison countries*' (i.e. Albania, Bosnia-Herzegovina, Croatia, Cyprus, Greece, Macedonia, Montenegro and Serbia) to stay in touch and let us know of any issues that they wish to raise with the EHC Steering Committee.

Thank you Naja and Miguel for introducing yourselves. The EHC is delighted to have you on board and wishes you a successful term.

**Naja Skouw-Rasmussen is a board member of the Danish NMO and a newly-elected EHC Steering Committee member*

***Miguel Crato is the President of the Portuguese NMO and a newly-elected EHC Steering Committee member*

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

*****Olivia Romero-Lux is an EHC Steering Committee member*

Meet the EHC Russian interpreters: Olga Miniuk and Julia Krivkina

Olga Miniuk and Julia Krivkina** interviewed by Laura Savini****

The European Haemophilia Consortium (EHC) has a number of National Member Organisations (NMO) for whom Russian is more common as a vehicular language than English. To better serve these NMOs and to include them in its activities the EHC has been using the services of two Russian interpreters, Julia Krivkina and Olga Miniuk for its Annual Conference, Leadership Conference, Inhibitor Summit and some of its workshops.

Besides interpreting presentations and discussions, Olga and Julia have also been of great help to EHC staff and Steering Committee members in communicating and interacting with this group of NMOs. As such, Julia and Olga are indirectly involved in the issues of these NMOs. Therefore, after many years of close collaboration we thought it was high time to give them a proper introduction to our readers and to learn more about how they came to work in this environment and the changes they noticed over the years.

Tell us about yourselves. How did you get involved in the haemophilia community?

Olga Miniuk (OM): I have been interpreting for the past 20 years and the first time we were brought to work in the field of haemophilia and other rare bleeding disorders was in 2008 for a regional workshop organised by the World Federation of Hemophilia (WFH). Subsequently we interpreted at the WFH World Congress held in Istanbul in 2008.

Olga Miniuk is one of the Russian interpreters for the EHC

Julia Krivkina (JK): I graduated from the University of St Petersburg's Department of English Language and Literature and my interpreting experience is over 30 years, so I can say I am a veteran!

OM & JK: After the conference in Istanbul, the WFH continued to require our services for regional meetings and workshops. In fact, Russian is a vehicular language not only in countries that belonged to the former Union of the Soviet and Socialist Republics (USSR) but also in some of the Baltic countries as well as Eastern European countries such as Poland and Bulgaria. The first time we worked for the EHC was in 2013 at the Annual Conference held in Bucharest, Romania.

How did you first learn about haemophilia?

OM & JK: When you have a couple of decades of interpreting experience, you have at some point worked in the medical field. However this is not a topic that one comes across often. Although now that we have been working in this area for a few years we have gathered some understanding of the issues and the disorder.

Naturally, being both Russian, we were familiar with the disease. In fact, the Tsarevich Alexi, the imperial prince of the Romanov family, was affected by haemophilia. We also had some of the common misconceptions that most people have about haemophilia such as that when people with haemophilia cut themselves, they can bleed to death, and so on. Obviously these myths were quickly dispelled when we started working in this field.

Have you noticed many changes in the haemophilia community since you first started working in this area?

OM: As we are involved professionally in this community, we are able to observe from a close distance changes that have occurred over time. For instance, the programmes are becoming increasingly comprehensive. As, in addition to the medical elements, there are a growing number of elements dedicated to community building and training for personal growth.

Julia Krivkina has been interpreting EHC events since 2013

JK: Furthermore, with regard to the scientific content of the events, the programmes and topics presented and discussed are becoming increasingly sophisticated. With regard to the Russian-speaking representatives of the NMOs, we note that they are more and more involved in the events so that they themselves are also becoming increasingly sophisticated. This is reflected in their knowledge and their interactions during these events. They have truly become the experts of their condition and have sometimes more knowledge than some of the clinicians in the room. This is of course more demanding for us as we need to increase our knowledge about bleeding disorders to be able to properly interpret their questions, which are no longer so simple. Furthermore, these patients have gained confidence and dare to ask questions. We really saw over time an evolution in some of the patients taking part in these events. Some of them used to be quieter and have progressively become bolder. In fact, they do not hesitate to be active, to ask questions and to interact during these events. Continuously taking part in these events has also definitely improved the knowledge of the Russian language for some of these participants. This has clearly made an impact as it allows them to be more at ease and more active. This truly showed us that language barriers can be very limiting to active participation in such events.

OM: We also noted that the information disseminated during these scientific and community-building events is clearly retained and used in their work. For instance, during the last Inhibitor Summit, one of the participants from a Russian-speaking country shared with us that inhibitors were not so well-known in her country. The same participant also explained that doctors in her country had been confronted with patients whose bleeds would not stop despite providing continuous factor replacement therapy. By gaining more knowledge about inhibitors during the Inhibitor Summit, this led her to wonder, in hindsight, whether this was due to the fact that these particular patients in her country were affected with inhibitors. Had these patients known about this complication, they could have perhaps asked the doctors to perform some tests or perhaps they could have asked for support through the haemophilia community in neighbouring countries. This truly showed us the power of knowledge. Now this patient knows what inhibitors are and when she will go back to her country, she will be able to share this knowledge with some of her colleagues and doctors. Knowledge empowers these patients to better look after themselves and their community as well as to advocate for better access to treatment and care.

JK: Throughout the years we witnessed patient representatives starting off as simple participants representing their country and then progressing to becoming expert patient advocates

representing their community at European or international level. On the other hand we also witnessed patients who attended these events at first but stopped attending once things improved in their country. This, from our perspective, is certainly a shame as both the scientific and medical knowledge as well as the tools for community building continue to evolve. So by missing scientific conferences and community building events, this probably leaves them with gaps in their knowledge. From our experience it seems that EHC NMOs should strive to maintain the community and continue to be involved not only during bad times but also during good times.

The EHC wishes to thank Olga and Julia for their first-class work and support! You will be able to meet them in person at the next EHC Leadership Conference, which will be held from 29th June to 2nd July 2017.

NMO News

NMO Profile: Macedonia

By Marija Nakeska, Board member Hemolog, the EHC Macedonian National Member Organisation

In each edition of the Newsletter of the European Haemophilia Consortium (EHC), we try to introduce our readers to one of our National Member Organisations (NMO) to learn more about their history, their current activities, challenges and plans for the future. In this edition, we hear from Hemolog, the EHC Macedonian NMO.

Dear Readers,

First I must say thank you for the opportunity to present our society in the EHC newsletter.

The bleeding disorder society of Macedonia – Hemolog - was established in March 1998 by three parents of children with severe haemophilia A. The main reason for setting-up this organisation was the lack of treatment in the country. In fact, at that time, the only treatment available in Macedonia for people with haemophilia was fresh frozen plasma (FFP). However, the founders of our organisation knew that there was a better way to treat their children and they decided to try to bring better treatment to our country. The first step was to contact the World Federation of Hemophilia (WFH), who offered them a treatment donation. However in order to be able to receive this donation, the organisation had to get established as a legal entity. Although this was the primary reason to establish the organisation, its founders knew that having a legal entity in the country would also facilitate advocacy towards access to better treatment and care.

The beginnings of our NMO were not easy and its founders encountered many problems both with government officials and with healthcare professionals who were not open to cooperation. That meant that they had lots of work but they weren't afraid of it. Founders Simon Atanasov, Zorica Furnadziska and Cvetanka Nakeska had different personal motivations but their goal was the same: better treatment for people with haemophilia in the country. Today, they are still active in the work of the society. After becoming WFH member in 1998, Hemolog became a member of the EHC in 1999.

Hemolog representatives

Today there are 213 people with haemophilia A in Macedonia and 107 people with haemophilia B. We also count 167 people with von Willebrand Disease (VWD) and 20 people with rarer bleeding disorders. The objective of our organisation is to represent all people with bleeding disorders and all those involved work towards this goal. At the moment 150 people are members of our organisation.

With regard to treatment and care, there is only one haemophilia treatment centre (HTC), which was established in 1999 as part of the Institute for Transfusion Medicine of Macedonia. In 2012, the HTC was relocated to new premises with separate entrance from the thrombosis centre, its own waiting room, musculoskeletal examination room and a room for psychological support. This treatment centre is certified as a Comprehensive Care Centre by the EUHANET project[3] and is also responsible for maintaining the national patient registry.

Celebrations during World Haemophilia Day

At the moment, our treatment consumption is three international units (IU) per capita, most of which are made up of plasmatic coagulation factor concentrate. The standard treatment regimen for adults is on demand and we have access to home treatment. Children have access to primary prophylaxis and adults have access to secondary prophylaxis during physiotherapy. Unfortunately, patients with VWD and patients affected by rarer bleeding disorders are still treated with FFP.

At the moment our organisation is going through some internal organisational changes, which we are confident will be beneficial for the future of the organisation. Our organisation is exclusively run by volunteers and of the seven board members, four are young men with haemophilia.

As much remains to be done, we have many priorities – and much enthusiasm! We try to be inclusive and represent all patients. Therefore we want to address generational issues and talk about disabilities. This would target 30+ year old individuals who are facing difficulties in finding employment and being socially included. We also want to create activities for women with bleeding disorders as we believe they should have equal access to treatment and care. We are also aiming to get novel therapies on the market and increasing the consumption of coagulation factor concentrate. A final, but crucial, point is the access to treatment for hepatitis C (HCV). As you can see, our dreams are big and we are hoping that our passion and hard work will solve most problems.

At the moment, our society organises many events, including annual meetings, workshops, summer camps, events to mark World Haemophilia Day, etc. However all of this work is dependent on the funds that we are able to collect. We work closely with other patient

3The European Haemophilia Network (EUHANET) is a project encompassing four elements, including the certification of haemophilia treatment centres in Europe. More information can be found at www.euhanet.org

organisations such as local patient organisations for HCV and AIDS as well as societies representing patients with disabilities. Finally we are one of the founders of the Alliance for Rare Diseases in Macedonia.

Hemolog representatives

As mentioned earlier, we are going through organisational changes. Our plan is to make smaller organisational units and to work with each separately. Last year, we created a support group for women, which aims to educate and support all women with bleeding disorders and carriers, but also those sharing their lives with someone with bleeding disorders, including partners. Two years ago we were part of the WFH Advocacy in Action (AIA) programme in order to reach out and diagnose patients with VWD, as a result, we are hoping that the women's support group will grow in the future. The next step is to create a youth group to engage with younger members.

I think that most NMOs in Europe are facing similar difficulties but for us the biggest challenges are funding and youth engagement.

Working for the NMO is always challenging and the most disappointing thing is when your work is not recognised for its actual value. That said, there are always people encouraging and inspiring you to do more and better. Although I no longer live in Macedonia, I am still working on the organisation and project administration but being not present makes it harder as I feel I am missing out on a lot of the activities of the organisation.

There are lots of proud moments that keep you going. This could be the smile of a mother with a newly diagnosed boy with haemophilia to whom you've offered comfort and support or when you see a person who used to be disabled and in a wheelchair walking again after having undergone orthopaedic surgery. I have been with the society from the very beginning and I clearly remember how things were when we started. This is why seeing progress, even if small steps forward, makes me feel very proud. For me the proudest achievement of our NMO is that it has managed to continue having the same inspiration as 18 years ago and keeping the same motto: 'Good deeds for no bleeds!'

News from Albania: Improving treatment through international cooperation

By Megi Neziri, volunteer with Shoqatës Shqiptare të Hemofilikëve, the EHC Albanian National Member Organisation

Megi Neziri tells us more about how her country, Albania, achieved higher treatment standards thanks to the generosity of the Italian healthcare system.

Albania is one of those countries that for many years couldn't provide adequate haemophilia care for its patients. At the same time the country was equally struggling to improve its own healthcare system. Unfortunately, the needs of haemophilia patients, in terms of factor replacement therapies, exceed the available resources. In addition, surgical intervention and prophylaxis couldn't be achieved.

Our mission has always been "treatment for all." First we fought very hard to convince our Minister of Health (MoH) that it was necessary to increase the amount of coagulation factor purchased every year. To do so we showed them that the cost of hospitalisation for people with bleeding disorders was higher than what it would cost to treat them. Unfortunately, we realised that even if we convinced our politicians, the increase in the haemophilia budget was still not going to be enough to provide adequate treatment.

Megi is a volunteer with the Albanian NMO

In 2015 we achieved our second goal, i.e. we finally got a National Haemophilia Centre at the Mother Theresa Hospital in Tirana, our capital city. Although the premises of the centre were ready as of 2013, it only became operational in 2015 when a clinician, a specialised nurse and a physiotherapist started their work there. Our National Haemophilia Centre greatly benefited from the cooperation it established with the Hospital Meyer in Florence, Italy, which since 2013 generously offered our centre 3 million international units (IU) of coagulation factor VIII each year with a commitment to continue for the next three years. In fact, Tuscany is one of the Italian regions with a large presence of Albanian citizens and the links between Tuscan Italians and the Albanian community are quite strong. Both have a strong commitment toward the improvement of the Albanian healthcare system. This programme was established in collaboration with the Tuscan chapter of the organisation International Health Cooperation.

What is encouraging is that Italian donors have renewed their commitment for another five-year period. This is thanks to a framework established under Italian legislation, which encourages excess FVIII to be donated to other countries. All of this has been made possible with the support of Kedrion, an Italian pharmaceutical company fractionating the plasma of Italian donors into finished product, i.e. plasmatic coagulation factor. This has allowed an increase in factor VIII use in Albania from 0.3 IU per capita to now 3.1 IU per capita. At the same time the MoH has not decreased the haemophilia budget.

We are happy to say that we have a HTC with good medical staff but what is even more important is that this centre is able to treat patients from all around the country. This has led to great

improvement in haemophilia care including prophylactic treatment for children above two years old and surgical interventions for people with damaged joints.

We still face many issues. For example we need educational programmes to explain to healthcare professionals that patients can do physiotherapy even if they are not under prophylactic treatment. We also have seen a rise in patients with inhibitors for which we do not have much experience and expertise. Nonetheless we are much more optimistic than ever before!

Our hope is that once the donation runs out, our MoH will be convinced by the many benefits of treatment and will give us an adequate budget for haemophilia in order to cover treatment for all.

Bloodrelatives: The end of a journey leading to the beginning of an adventure

By Evelyn Grimberg, volunteer with Nederlandse Vereniging van Hemofilie-Patiënten (NVHP), the EHC Dutch National Member Organisation

A team of volunteers from the EHC Dutch National Member Organisation (NMO) decided to cycle more than 1,000 kilometers from Utrecht to Stavanger to raise awareness about the importance of sport for people with bleeding disorders. Evelyn Grimberg, a young volunteer with the NMO describes the experience, lessons learned and next steps.

The cycling team 'Bloodrelatives'

After a year of preparation, the 'Blood Relatives' cycling team of the Dutch NMO was finally set to start its 1,000 kilometres journey to Norway. This cycling trip would bring us from Utrecht, the Netherlands to Stavanger, Norway to attend the Annual Conference of the European Haemophilia Consortium (EHC). The journey would take two and half weeks including 11 cycling days.

The purpose of the trip was to create awareness within and outside of our society. We wished to make people realise the importance of sport to keep your body physically healthy. We also wanted to raise money for the society. The big question, now that our journey is completed, is to know whether we have achieved our goals. It is time to evaluate the experience.

During the year preceding the experience we organised several activities to raise awareness and involve our members in this project. During World Haemophilia Day we organised a bike trip to which all members of our society were invited. We held a 24-hour bike challenge during our family camping weekend. All the parents and some children were game and took part in the challenge. We also gave presentations both at the treatment centres and during our annual meeting. We engaged with pharmaceutical companies to inform them of our work. We developed a Facebook page to keep tally of all of our activities but also to ask our members and the general public to get involved. Our objective was to encourage our members to take part in monthly exercise

challenges. The feedback was positive and we noticed that many of our members took part in these activities. Even older members told us that they became more active by starting doing exercise.

At first we found it quite difficult to publicise our project outside of our haemophilia society. However, we were able to get in touch with the media thanks to the support of a communications company. Our story caught the attention of regional media and it resulted in radio interviews, articles in newspapers and online posts on websites. In total our campaign was picked up by media outlets with the potential to reach a range of 5,000,000 readers and listeners. This was more than we could ever have dreamed of!

Dutch NMO President, Ad Schouten (left hand-side) and Dutch NMO member Chris van den Brink provided technical support to the cycling team throughout their journey

Secondly we set up a special donation website where people could support us with financial donations. In total we were able to raise €7,000, which we are planning to donate to our society to support them but we are also planning to give some of this money to other European societies to support awareness projects related to the practice of exercise and sport. We also want to develop a leaflet with some key information on bleeding disorders and sports.

As we rode through Europe to a European Congress we wanted to emphasize the international aspect of our project. During our trip we met with members of the German haemophilia society and one of their youth volunteers cycled with us for a day. While in Denmark, we met with the local haemophilia association and visited the treatment centre in Arhus. It was extremely valuable to meet other patients and share experiences but also to see and experience how another treatment centre works.

This outcome, i.e. many people affected by bleeding disorders cycled more than a 1,000 km each, could not have been achieved without a lot of hard work. We had to train our bodies to get used but also to get ready for the many kilometres we were to cycle. We were able to do this also thanks to the support of the van Creveld Clinic in Utrecht. We really wish to thank all the medical staff that helped us on our journey. In fact the clinic provided us with three sets of tests to evaluate our physical condition, the status of our muscle mass and our dietary habits. To improve all of this they assigned us a dietician and sports specialist who followed us throughout the year. The tests were ran three times throughout the year to assess progress and to ultimately evaluate whether we were ready for the trip.

However, physical preparation was not the only thing that we had to look after prior to our journey. A lot of time and effort was spent publicising the project, building and updating a website and social media and last but not least organising the practical details of the trip itself. Our team members were truly committed to the project. We met every month to discuss the progress. As with every project, we soon realised that the amount of work involved in developing this was bigger than expected. For instance, we had to find sponsors, collect the right material and many other small but time-consuming details that had to be adjusted prior our departure. All of the

team members did this as volunteers, which meant that they still carried out their '*normal*' life on the side. However, we knew that we had to dream and think big to achieve our set goals and make this project a success. We are extremely proud of our achievement and really wish to thank all those that have supported us along the way.

EHC Conference delegates gave a warm welcome to the cycling team

Nonetheless, the project did not finish with the cycling trip. We intend to continue our campaign on the importance of exercising and physical activity. We wish to expand 'Blood Relatives' and organise a national meeting in the new year to highlight this message. We are also planning to continue our outreach to local media.

Finally, this trip could not have been a success without the support of many of you, dear readers. The warm welcome you gave us in Stavanger was overwhelming. It was truly an emotional moment to see all the happy proud faces. To see many friends who so warmly welcomed and embraced us was priceless. The award ceremony that you organised during the General Assembly meeting was superb. We want to thank everyone for following our journey and supporting us. In particular a special thanks to the Norwegian association and the EHC for the great welcome at the conference.

International Basketball Camp for People with Haemophilia 2016

By Egidijus Šliaužys, President of Lietuvos Hemofiljos Associja, the EHC Lithuanian National Member Organisation

From 8-14 August 2016 orange basketballs shot through the hoops at the sports centre and outdoor basketball courts in Birštonas. The Lithuanian Association of Haemophilia organised the first ever International Basketball Camp for People with Haemophilia. Guests that came to enjoy the natural beauty of this resort town, to have fun playing games and play on the basketball court, were not only from Lithuania but also from Bulgaria, Poland and Belarus.

The camp programme consisted of daily intensive basketball workouts, exercising in the gym and in the swimming pool, lectures on healthy lifestyle, the advantages of sports for people with haemophilia and news regarding bleeding disorder treatments.

Children were trained by Indrė Keršulytė, Eglė Žibinskaitė and Ieva Stankevičiūtė, coaches of the Lithuanian Association of Haemophilia basketball team. These ladies worked very professionally by taking into proper consideration the physical condition of each child and recommending adapted physical exertion on an individual basis. Each training and workout began with warm-up exercises. Warming-up is very important for people engaged in physical activities such as sport because it helps to avoid injuries and traumas. The coaches were very happy to see a serious approach to workouts from participants with quick daily improvements. The final results of the basketball game showed that men were taking their training seriously. Even younger newcomers to basketball learned some skills and got to know the sport known *assecond* meaning *religion* in Lithuania. But the most important thing is that they gained trust in themselves and had a chance to see that the disease is not an obstacle to participating in sports.

Participants at the Lithuanian NMO international basketball camp, in red the Lithuanian NMO basketball team and in the grey the H Force team

In the Tulpės Health Centre, Viktorija Jušinskaitė, a specialist in physiotherapy, conducted indoor workouts and exercises in the swimming pool. She applied knowledge that she had obtained working with patients with haemophilia and employed different physiotherapy approaches and exercises. Although the training was conducted in groups, those who were not able to do some of the exercises were encouraged to do alternative ones with more appropriate exercises.

Not all participants who arrived at the camp knew how to administer medications. Onutė Malinauskienė, a nurse from the Tulpės Health Centre, helped them to overcome this barrier. Parents that came to pick up the participants afterwards noted how their sons became robust and self-reliant.

When you put effort and energy into something, you want to see a result. The intensive sports training week concluded with a friendly match between Lithuanians and foreign guests organised in the sports centre of Birštonas high school. Players from the Lithuanian Association of Haemophilia basketball team played against the H force team that had been put together during the camp. The Lithuanian side won the competition.

The key goal of the International Basketball Camp is not about the number of steals or three-point shots. The true meaning emerges when you see the men who suffer from haemophilia playing on

the court. On the day of their departure, they were happy after having spent a week in Birštonas. Most of them said they didn't want to go home and dreamed of returning here next year.

Comments from the participants:

By Bartek Andrzejkiewicz from Polskie Stowarzyszenie Chorych na Hemofilię, the EHC Polish National Member Organisation

Bartek, a member of the Polish NMO attended the Basketball camp

First of all, this camp gave us a chance to meet patients with haemophilia from Lithuania, Belarus and Bulgaria. Although it may seem paradoxical, we met colleagues from Poland as well. In our country, such meetings with colleagues are only possible at a regional level. We could say whole-heartedly that Egidijus had organised something extraordinary. He managed not only to overcome the stereotypes of interaction between different nationalities but busted the myth about our disability as well. He showed that we can be in good physical condition and feel good, despite many constraints.

Participants working out in the swimming pool of the Tulpės Health Centre

After a week of workouts in the gym, swimming pool and basketball court, we were rewarded with a basketball match between our international team and the team from the Lithuanian Association of Haemophilia. Well, we lost the game, but we didn't feel like losers! We sweated it out on the court, put our hearts into the game, and played our role in full. The final whistle announced our accomplishment. We had been striving for this inner achievement from the moment we arrived in Lithuania.

Irreplaceable coaches, a specialist in physiotherapy and Egidijus made a great contribution to our success. They were constantly encouraging us, spared no good words and were happy for our small victories. They created a wonderful atmosphere not just in terms of sports competition but an atmosphere that inspired inner strength.

Ryszard Basara from Polskie Stowarzyszenie Chorych na Hemofilię, the EHC Polish National Member Organisation

Eight people from Poland registered for this camp. This was the biggest group of guests. The team of the Lithuanian Association of Haemophilia was also among the participants. It was great to meet blood brothers from other countries (Bulgaria, Belarus and Lithuania) as well as from

Ryszard, a member of the Polish NMO attended the basketball camp

different Polish regions. Even though we all were of different age (I am 33 years old, the youngest boy was just 15) and we spoke different languages, we soon teamed up.

We had three very energetic coaches, Indrė, Eglė and Ieva. These ladies are professional basketball players who train the basketball team of the Lithuanian Association of Haemophilia. We started every workout with warm-up exercises. Then we learned proper shooting technique, how to make accurate passes to team members and trained these skills. If someone wasn't able to do certain exercises, he was encouraged to do more appropriate exercises. Not all of us with haemophilia are in good physical condition. Therefore, we must put in extra effort in order to do what a healthy person does easily. Each of us tried to do our best with every sports task, and supported and encouraged other participants because in the end team spirit matters most. We had workouts not only on the basketball court but in the sports hall of the Health Centre and in the swimming pool as well. Viktorija, a physiotherapist, conducted these workouts. Egidijus, the organiser, took care of all our needs, including timely provision of medical assistance and kept us enthusiastic and in a good mood.

We were also greatly impressed by the Olympics held in Brazil at the same time as the camp. We watched the basketball competitions with excitement together with Lithuanian colleagues and supported the Lithuanian national team with eager enjoyment. At the end of the wonderful week, we had a big game between the team of the Lithuanian Association of Haemophilia and guests from the foreign national basketball team under the new name *H basketball force*. This name sounds quite threatening and proud. We were very proud of challenging Lithuanian colleagues who play basketball more professionally. Perspiration poured on the basketball court; we tried to fight to the finish by employing all the skills we had learned during the training. Each player properly performed game elements, shots were praised by audience applause, which gave us great inner satisfaction. Even not very successful moments received support from colleagues and gratitude for the courageous attempt. We had a friendly atmosphere on the court during the competition, but the more experienced Lithuanian team won the game by a score of 52:42. Despite this result, each of us felt like a winner.

Participants during the final basketball match

To summarize the impressions of this week spent by the beautiful Nemunas river, this was one of the most interesting weeks in my life. Even today I continue humming the words from the Czeslaw Niemen song *"...time flows like river, like river, taking away these days to the past..."* This was a super adventure for me with new wonderful people, professional basketball, the Lithuanian language, national cuisine and nature.

I recommend everyone to participate in this educational basketball camp next year that is intended for patients with haemophilia in Lithuania! I hope to meet new friends in the future. I am once again very grateful to the event organizers for my experience.

German NMO elects a new president: Meet Stefanie Oestreicher

By Stefanie Oestreicher, President Deutsche Hämophilie Gesellschaft, the EHC German National Member Organisation

Stefanie is the new President of the German NMO

Dear EHC community,

At the end of October I was elected as the new president of our German Haemophilia Society. I am thankful for the sound support I received from our members. At the same time, I view this nomination as a high responsibility.

For those who don't yet know me, let me shortly introduce myself. I am 44 years old and married. My husband has severe haemophilia A. From my professional background, I have a Masters' degree in biomedical engineering and computer science. I received a PhD in bone structure research and have many years of experience leading international teams and departments in the private sector. Since 2013, I have been on the board of our society responsible for EHC and WFH affairs. In my private life, I love to travel and sail on lake Constance, located on the border with Switzerland, Germany and Austria.

I am looking forward to the challenges of my new position. However, I am also conscious that I have the opportunity to take over the presidency at a very exciting phase. There are so many new treatment alternatives under development and the treatment of haemophilia is about to change significantly. Providing access to our patients is a very important goal. In this context I am happy to have the great support of the EHC at a European level.

At the same time, our haemophilia society is also at a crossroad. On the one hand, we have to follow our heritage and in Germany we are currently fighting for the continuation of the HIV compensation. Fortunately, the political signs are going in the right direction. On the other hand, our young members have a significantly different focus. Thanks to a strong youth board, which was established more than 15 years ago, we have a good overview of their interests and needs. After a successful activity last year held to mark World Haemophilia Day, which was also acknowledged by Alain Weill, President of the World Federation of Hemophilia (WFH), at the WFH Congress, our youth is currently planning a follow-up activity for the next World Haemophilia Day with high enthusiasm. Nevertheless, both the interests of the ageing haemophiliacs, as well as of our young parents, have to be respected. Servicing our members with their individual needs remains the main focus.

Yours sincerely,

Dr Stefanie Oestreicher

The EHC wishes to congratulate Stefanie on her new position and wishes her much success in her presidency.

Changes within the EHC Swedish National Member Organisation

By Monika Westerberg and Therese Backus***

Earlier this year Förbundet Blödarsjuka i Sverige, the Swedish National Member Organisation (NMO) of the European Haemophilia Consortium (EHC) elected a new President. Additionally, the NMO also hired a new director to manage its day-to-day activities. In this article we briefly meet Monika Westerberg and Theresa Backus who tell us a little more about themselves and their plans for the future of the NMO.

About Monika

I would like to introduce myself as the newly elected president of Swedish Haemophilia Society (FBIS). I'm really proud and honoured to be the first woman elected to this post. My name is Monika Westerberg and I'm 43 years old. I live on a small farm outside Gothenburg with my husband, three kids, three dogs, three horses and a cat. I work full-time on an outdoor pre-school as a teacher.

Monika is the new President of the Swedish NMO

Eleven years ago my eldest daughter was diagnosed with a bleeding disorder named ITP (Immune Thrombocytopenia Purpura). She was eight at the time when we noticed a lot of bruises on her body. We then went to the doctor and found out that her platelet count was really low. At first we thought it was leukemia but thankfully it was just ITP. A normal platelet count is around 150-400 billions platelets/litre and when you have had lower than 150 for over a year, it's what is called chronic ITP. At first we hoped for an acute ITP but she wasn't recovering and today she is still living with this condition. We didn't get a lot of information at the time and the doctors didn't seem to take the condition seriously (probably because we were at the leukemia centre and they saw really sick kids every day). It made me so frustrated because I saw that my daughter didn´t feel well at all.

ITP is an autoimmune disease and its causes are still unknown. Probably one cause is that the patient had a virus and the immune system attacks the platelets because it's mistaking them for a foreign body. Spontaneous bruising, petechiae[4] and heavy menses are some of the signs of ITP. More severe and rarer symptoms include blood blisters in the mouth, blood in the urine or stool and intracranial bleeds.

Ten months after my daughter's diagnosis I came across the Swedish Haemophilia Society and they told me about an ITP conference coming up in the next few weeks. That conference was so important for us! We were able to talk with other people affected by the disease, to hear their stories and their advice on how to cope with everything. After that weekend I felt a lot better with the disease. In Sweden ITP patients are represented by the Haemophilia Society because of their symptoms, which involve bleeds. It is at that time that I started to become involved in the society's activites. For the past eight or nine years, I have been involved in the NMO ITP committee as well as in the NMO regional board. The ITP committee develops yearly conferences focused on ITP, which are really important because this is, after all, a rare disease and so it is hard to connect with other people affected by the same disorder. Since the beginning of my involvement with the

4 Petechiae are small red and purple spots on the skin cause by bleeds.

Swedish NMO, I have also travelled internationally to meet with other ITP associations. We have also developed an international website for those who wish to get more information about this disorder: http://www.globalitp.org.

My involvement with the society meant that I have also met many people with different bleeding disorders. This really gave us the opportunity to meet a lot and make new friends. For me it is very important to be able to meet with people facing similar challenges and to share experiences. My daughter and I have also attended meetings of the NMO women's group that has been running a really exciting project for the past three years. It is really important to find girls suffering with different bleeding problems and to enable them to share their experiences and meet with peers facing the same challenges.

This summer I had the opportunity to travel to the congress of the World Federation of Hemophilia held in Orlando, USA. It was really interesting and I learned a lot. This experience really got me thinking about getting involved in our NMO board. This is in fact the right time for me as my children are getting older and I feel very passionate about the work of the NMO. I have a lot to learn about being president but I am very motivated and look forward to this role!

At the moment our NMO is moving office, which many of you will know is a lot of work. Next year we plan to hold the Nordic meeting in our country and are getting ready to host it. Another big challenge ahead of us is to get all of our members with hepatits C (HCV) to access treatment. We also publish a newsletter, *Gensvar*, four times a year. Every year we also organise a lot of activities like family conferences, feel-good days for our elderly members, a meeting for women and the ITP conference. We also intend to contine the international collaboration with fellow countries.

Finally I would like to say that I'm really looking forward to the next two years on the board. We work together to make a good society for our members with a lot of help from our staff at the office.

Therese joined the Swedish NMO in April 2016

About Therese

My name is Therese Backus and I am the new director of the Swedish Haemophilia Society. I worked for the Swedish NMO from 2011 until 2014 and I have recently (April 2016) joined the association again. In my new role, I have overall responsibility for all functions in our organisation. I am pleased to say that our NMO has a very good team. Our board is strong, hard-working and committed to the goals of the organisation. The board is renewed every two years and works with the NMO on a volunteer basis. We currently employ two staff members: Sofia Nordell and Maneka Ghosh. They are mainly working with us on our women's project, which aims at raising awareness about women also being affected by bleeding disorders. In fact, we believe there are many people who have a mild form of von Willebrand disease (VWD) and other bleeding disorders who are not identified. This leads to situations in which both men and women are facing difficulties in accessing correct diagnoses.

I have a lot of experience in working in the volunteer world. In fact, many of my family members were involved as volunteers in different organisations. For my part, I volunteered a lot with the Swedish church when I was younger. In fact, their children and youth programmes are probably

the best in Sweden. Other influential volunteer organisations in Sweden include sports clubs and the disability movement.

I have a Bachelor degree in political science and a background in working in youth programmes both in church organisations but also in other patients' groups. Although I had no connections to haemophilia prior to this job, I felt I had the right background for the job. Additionally being a woman I feel very connected to the women's project and believe it is important that women are able to obtain the right diagnosis. I also felt very touched by the contamination tragedies and the stigma surrounding people affected by HIV. Unfortunately, of the 104 people in the haemophilia community that were contaminated with HIV through their treatment, only 30 are still alive. We organise a training weekend on research, health and haemophilia for this group every year.

One of the challenges of our community is the 'invisible' disability affecting our members and the limited knowledge that the general public has about this condition. Another major problem is inhibitor development as this condition is extremely painful and straining. Although we have extremely good healthcare and welfare systems in Sweden, we are vigilant that these systems do not disappear.

Our organisation has about 1,600 members representing individuals affected by haemophilia, VWD, ITP, other bleeding disorders as well as family members. At the moment in Sweden we have some 1,000 people affected by haemophilia, 1,500 affected by VWD and 600 people with other rare bleeding disorders. Almost half of these patients are not yet our members, so one of our challenges is to widen our membership base. Another challenge is to get people aged 30 to 45 to remain active in the organisation. This is because this is the age where they typically start to have their own families, although we see them coming back especially if their children are carriers or affected by a bleeding disorder.

One of our biggest activities is our national education camp where we gather members from all over Sweden. We also have training weekends and camps for all age groups. Our members are very well trained and have much expertise about their own disorder. We also support families with newly diagnosed children so they can exchange experiences. Another one of our major areas of focus is to get treatment to those affected by HCV. Of the 400 individuals from the bleeding disorder community who became infected with HCV in the 80's, about half are still infected. We are demanding that every patient affected by HCV receives HCV treatment irrespectively of their liver damage

I am very happy to be back with the Swedish NMO as I greatly enjoy the people and issues I work with.

Many thanks Monika and Therese for these introductory words. The EHC wishes you much success in your work with the Swedish NMO.

**Monika Westerberg is the President of the Swedish NMO*

*** Therese Backus is the Director of the Swedish NMO*

Feature Articles

Researcher Spotlight: Karen Vanhoorelbeke

Karen Vanhoorelbeke interviewed by Laura Savini***

For this edition of researcher's spotlight we interview Prof Karen Vanhoorelbeke, a molecular biologist by training working at the Catholic University of Leuven in Belgium. Karen's research interests include von Willebrand Disease (VWD) and thrombotic thrombocytopenia purpura (TTP).

1) What are you currently working on? What is your research area?

Karen Vanhoorelbeke is a molecular biologist at the University of Leuven, Belgium

During my post-doctoral studies I started working on von Willebrand Factor (VWF) and platelets and in 2001 the ADAMT13 enzyme was discovered. This is an enzyme that is responsible for cleaving VWF into smaller units. People that have a deficiency of this factor are affected by TTP[5].

At first my research interest was the study of ADAMT13 because at the time it was discovered we literally didn't know anything about it. My team and I wanted to understand its structure, its function, its inhibition and so on. As mentioned, this research led to the study of the disease caused by ADAMT13 deficiency: TTP. We wanted to learn about the disease's pathophysiology. We researched and developed new diagnostic assays as well as animal models and tested novel medicinal products for this condition in animal models. This is really translational research, meaning that we start from fundamental knowledge and we see how it can be applied to develop new tools (e.g. diagnostics or medicinal products) to improve patients' lives. For instance a few years ago, we were able to develop a new diagnostic tool for VWD, which has now been commercialised and used in clinical settings.

My passion for molecular biology has also led me to carry out some work on gene therapy in the area of VWD. A few years ago a new non-viral technology for gene therapy, called chimeraplasty, was developed and we spent a lot of time working with this technology by performing many in vitro experiments. Unfortunately the technology did not live up to its promises and eventually it was concluded that it was not effective in gene therapy. This is unfortunately one of the downfalls of working in research. Today, however, we are continuing this research on gene therapy in VWD by using both viral vectors and non-viral strategies. However we are still very much in the early stages.

5 TTP is a rare disorder of the blood coagulation system in which microscopic clots form through the body. If left untreated these clots can damage organs such as the kidneys, heart and brain. The cause of TTP is the inhibition of an enzyme ADAMT13, responsible for separating VWF into smaller units.

2) What does your average day involve?

My days are very diverse. I manage a team composed of PhD students, one post-doctoral researcher and lab technicians. So there is a lot of coordination to be done, checking and analysing data, discussing new ideas, supervising and guiding my students. As every researcher will know, a lot of my time is also devoted to preparing grant applications to finance our work. Another aspect of my work is to ensure the dissemination of our work and sharing knowledge with other experts. This includes teaching but also preparing scientific papers and interacting with other scientists by attending conferences and giving presentations. I am involved in several international networks through my participation in the International Society for Thrombosis and Haemostasis (ISTH), the European Haematology Association (EHA) and the newly established European Congress for Thrombosis and Haemostasis (ECTH). Sharing information and collaborating with colleagues at an international level is very important to further research. As I do not work directly with patients, another important part of my work is to work closely with doctors who are directly caring for patients. Though them we get not only patients' samples but also their feedback on the progress of their medical condition.

3) Why is your work important? What do you hope the impact of your work will be?

I think my work is important because I have the opportunity to train students and to support them in becoming excellent researchers with a broad range of skills, not only related to scientific research.

Also, I am hoping that through my research and the fundamental knowledge that we uncover, we can improve diagnosis and treatment of people affected by TTP and VWD. I am hoping that this will ultimately result in new treatments to improve their quality of life.

4) What keeps you awake at night?

There are two things that keep me awake at night. The first are questions arising from the research that we carry out. For example if we uncover unexpected findings, I will be wondering about their meaning but also if we are facing challenges I will be wondering about how to overcome them.

The second question is how to keep-up the enthusiasm and motivation of students throughout their PhD or post-doctoral training.

5) Tell us one thing that you learned about bleeding disorders that really surprised you.

It was not so much a specific fact about VWD or TTP that impacted me mostly but it was meeting actual patients and realising that they need treatment. This gave a human dimension to my work and made me see the potential and impact that our work can have.

6) How did you become involved in this field and on this topic?

As with many things in life, it was a mix of opportunities and research interests that led me to my current position.

7) What is the most frequently asked question about your work?

People who are not involved in research are really surprised by the amount of time and effort needed to carry out research.

8) What is the next big thing that is coming in your field of work?

This is more related to my work in TTP. TTP has an acute phase but then after the patient is treated he or she can relapse and we do not know why and when. Doctors need to understand what triggers this relapse so that treatment of these patients can improve.

9) If you had not been working on this topic, you would have been working on…?

I would be probably be working in neurobiology.

10) What would be your advice or recommendation to someone with a bleeding disorder?

I would encourage people affected by a disease or disorder to try to understand as much as possible about their condition and to talk not only to doctors but also to researchers to understand the underlying causes of their medical condition. This will also help them to better understand what is being done to identify and develop novel treatments. On the other hand, I think it's key for researchers to not only disseminate their research to colleagues but also to ensure that it is translated into lay language so that those affected by these disorders can learn more about their condition.

** Karen Vanhoorelbeke* is *a molecular biologist at the Catholic University of Leuven*

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

Results of patient surveys concerning their expectations and needs vis-à-vis the new up-coming Extended Half-Life (EHL) products among haemophilia patients and their parents from the DACH region

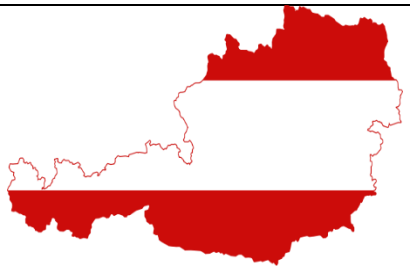
By Dr. Sylvia von Mackensen, University Medical Centre Hamburg-Eppendorf, Germany, on behalf of the Deutsche Hämophilie Gesellschaft (DHG), the EHC German National Member Organisation (NMO), Schweizerische Hämophilie-Gesellschaft (SHG), the EHC Swiss NMO and Österreichischen Hämophilie Gesellschaft (ÖHG), the EHC Austrian NMO.

New extended half-life (EHL) products for treatment of haemophilia A and B have already been licensed in some countries or will be available in the near future. These treatments are expected to improve patients' quality of life and to increase treatment adherence. However we don't know much about the patients' perspectives on the use of these new EHL products. We planned to extend the survey we conducted among the members of the DHG in Germany in 2015[6] to all PWHs registered with the EHC Swiss and Austrian NMOs in order to retrieve data for the entire DACH region[7].

6 See EHC December 2015 Newsletter.

7 The DACH region encompasses **Germany (D), Austria (A) and Switzerland (CH).**

Some facts about the DACH Region

Germany has 82 million inhabitants, of these around 8,200 have haemophilia, with an estimated 6,900 individuals with haemophilia A and some 1,300 with haemophilia B. 6,228 patients are included in the German haemophilia registry with 3,730 individuals affected by haemophilia A and 706 individuals affected by haemophilia B. Therefore 54 per cent of the expected haemophilia population is documented in the national registry.	
Switzerland has 8.3 million inhabitants with some 800 individuals affected by haemophilia with an estimated 700 individuals affected by haemophili A and 100 individuals affected by haemophilia B. 967 patients are included in the Swiss haemophilia registry, of which 704 individuals have haemophilia (587 with haemophilia A and 187 with haemophilia B). This means that approximately 88 per cent of the haemophilia population is documented in the registry.	
Austria has 8.47 million inhabitants with an estimated 847 individuals affected by haemophilia. It is estimated that 720 people are affected by haemophilia A while 127 individuals are affected by haemophilia B. 862 individuals are recorded in the Austrian haemophilia registry including 753 people with haemophilia (of which 635 have haemophilia A and 118 have haemophilia B). This means that 89 per cent of the expected haemophilia population is documented in the registry.	

NMOs from the DACH Region were interested in better understanding the expectations of people with haemophilia (PWH) as well as their concerns regarding these new EHL products. Furthermore, these organisations wished to understand to what extent PWH would be willing to switch to these new products based on their half-life extension. Finally, we wanted to learn from these findings about the gaps in understanding of these new treatments. This was so that NMOs could adapt their support of their respective patient community by developing tailored information tools such as leaflets, newsletter's articles, etc.

The survey included questions concerning the following aspects:

1. Demographic and clinical data,
2. Knowledge about half-life of current factor concentrates,
3. Attitude towards factor concentrates, including:
    a. satisfaction with current factor concentrate,
    b. expectation vis-à-vis new long-lasting products,
    c. willingness to switch to new long-lasting products,
    d. required information,
    e. information sources,
    f. facilitation of therapy.

In total the survey was sent to 2,644 PWHs and 1,012 surveys were sent back (a total of 38.3 per cent response rate). The response rate was broken down as follows:

- Of the 1,499 individuals contacted in Germany, 697 responded, equalling to a 46.5 per cent response rate.
- Of the 658 individuals contacted in Switzerland, 221 responded, equalling to at 33.6 per cent response rate.
- Of the 487 individuals contacted in Austria, 94 responded, equalling to a 19.3 per cent response rate.

Of the received 1,012 responses only 1,007 questionnaires could be analysed as five patients had other bleeding disorders and therefore were not a relevant target group for the survey. From the 1,007 valid questionnaires, 743 were from patients and 262 from parents of children with haemophilia. 84.5% of the respondents were affected by haemophilia A, 73.7 per cent of the respondents were affected by severe haemophilia, 57.8 per cent of the respondents were on prophylactic treatment and 60.2 per cent of the respondents used recombinant products. About a quarter of the respondents did not know the correct half-life of their current treatment. This is in line with some of the respondents' statements regarding the expectations towards new products, including: "*It would be great to know what is meant with half-life. I have a rough idea, but I don't know it* (from a German respondent)" or "*Until today unfortunately I did not know what half-life is, I will inform myself quickly* (from a German respondent)" or "*With my actual knowledge about factor concentrates it is not possible to give a realistic evaluation* (from a Swiss respondent)" or "*I would need more information in order to have specific expectations*" (from a Swiss respondent) or "*No deterioration*" (from an Austrian respondent).

In general, most of the PWHs were **satisfied with their current factor concentrates** (see image below).

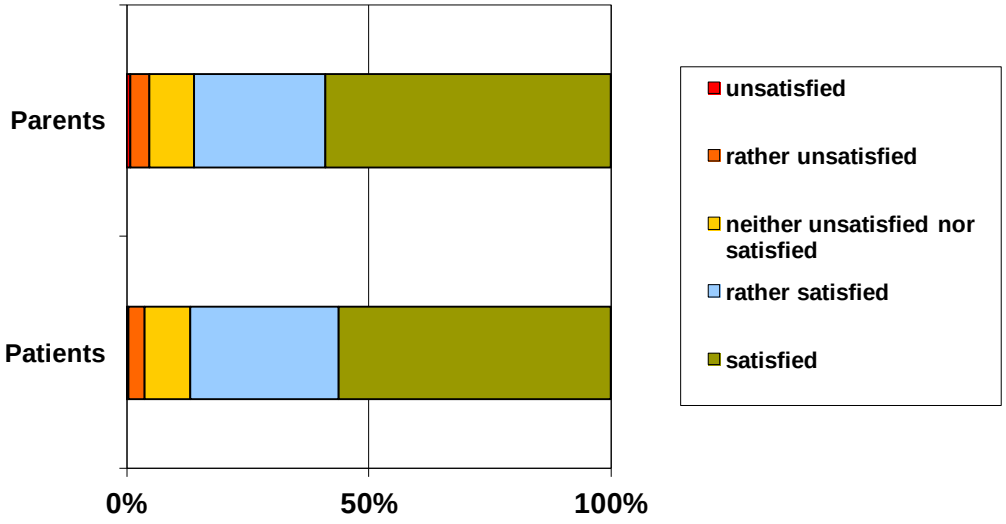


Figure 1: Satisfaction with current factor concentrate of participants (parents, adult patients)

They mainly expected from new products less frequent injections, efficacy and safety of the product. Some PWHs had a quite positive attitude towards the new products, which is reflected in the following statements: "*I absolutely want to have the new factor concentrates*" (from a German respondent) or "*A dream for kids*" (from a German respondent) or "*To have a better life*

– *mentally and emotionally!*" (from a Swiss respondent) or "*After substitution I can be away a couple of days without worries*" (from a Swiss respondent) or "*Improved quality of life since child has not to be injected so often*" (from an Austrian respondent).

PWH would be **willing to switch to** new products if these products had a prolonged half-life, the same safety profile of the current factor concentrates and were travel-friendly. More adult patients would be willing to switch to new EHL products compared to parents of children with haemophilia who were more skeptical (see image below). **Reasons for not be willing to switch** were the fear of inhibitors and uncertain regarding the safety of new products.

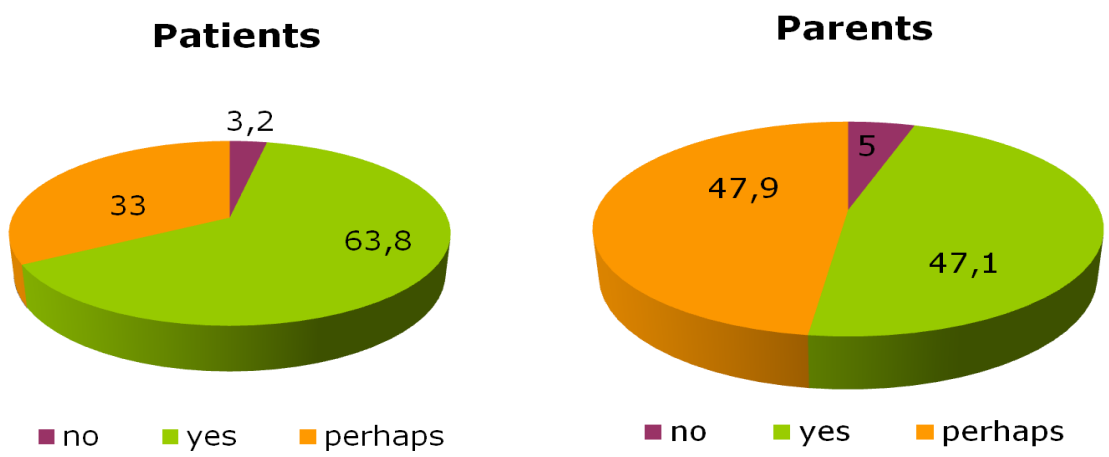


Figure 2: Willingness to switch to new longer-acting products (adults, parents)

PWHs from the DACH Region had almost no information about the new up-coming products, but **wished more information** about half-life, possible side-effects and efficacy. 40.5 per cent of the respondents would consider switching products if the **prolongation of the half-life** is at least double the one of their current factor concentrate.

Most PWHs wished to be **informed about** new products from their haemophilia treater, their NMO and joint information letter from both their haemophilia treatment centre and their NMO.

The **biggest difference** in responses in the surveyed countries was the willingness of parents to switch products for their children. Parents in Austria and Germany are more conservative compared to the adult patients. In Switzerland the difference in response between parents and adult patients was minimal as both groups were more willing to switch products.

Conclusion & Outlook

- PWH from the DACH Region were generally satisfied with their current factor concentrates. Those who were unsatisfied complained about short half-life, difficult manageability and storage conditions of their current factor concentrate.
- The findings of this representative survey among PWH from the DACH Region showed new insights on how PWHs want to be informed about new products. The survey also shows us which information they require to be able to make an informed decision with

regard to changes in their treatment. We also found out from whom these respondents wish to receive this information.

- The majority of respondents did not have a lot of information about the new EHL products, but would be willing to switch from their current factor concentrate to these products assuming the half-life is prolonged and has the same safety profile as their current factor concentrate.
- Based on these findings the DHG has already developed an independent patient presentation regarding the new technologies of EHL products in cooperation with members of its medical advisory board, which has been distributed to haemophilia treaters in order to comprehensively inform their patients about new technologies.
- Currently a patient brochure on these new EHL products is being produced with the information required by PWH and will be sent out via the NMOs of the DACH Region.
- Results of the national surveys have been presented at the respective NMO meetings and were published in their respective NMO newsletters (DHG: “Hämophilie Blätter”; ÖHG: “Faktor Magazin”; SHG: “Bulletin”).
- The results of the DACH Region have been submitted to national and international scientific congresses and a publication has been submitted to an international journal.
- The findings are very helpful to support the preparation of information material on the new and up-coming EHL products. In fact, they help us understand our members’ needs regarding which information they wish to receive.
- We believe that it could be a real opportunity to repeat the same initiative in other countries to see whether PWH have the same expectations and needs vis-à-vis the new up-coming EHL products. To implement a similar questionnaire in other countries would help patient associations and physicians to better inform patients about the new up-coming products. In our opinion this understanding of different patient needs from various countries will facilitate the work of patient associations in these countries to support their members appropriately in decision-making.
- If you are interested in conducting a similar survey in your country we would be pleased to provide you with the questionnaire, which can be adapted to your country’s needs in cooperation with us. Please feel free to contact us under the following email address **s.mackensen@uke.de**

News from our corporate partners

The EHC would like to acknowledge and thank the 2016 corporate partners of the EHC Round Table of Stakeholders programme, which promotes dialogue and exchange between all stakeholders. In this spirit we are pleased to share their news with readers in this section.

Platinum partners:

- Novo Nordisk
- Pfizer
- Roche
- Shire
- Sobi

Gold partners:

- Bayer
- CSL Behring
- Grifols

Silver partners:

- Biotest

Together we are driving change in haemophilia

We believe improved joint health and mobility are important for people living with haemophilia. Through our Talking Joints® educational programme and the Novo Nordisk Haemophilia Fitness Camp, we want to support improving knowledge of joint care.

Together with our research programmes and HERO grants*, these are some of the many ways we are changing haemophilia.

Learn more about our commitment at **novonordisk.com/changinghaemophilia**

*HERO grants is an unrestricted grant programme supported by Novo Nordisk Healthcare AG

changing haemophilia™

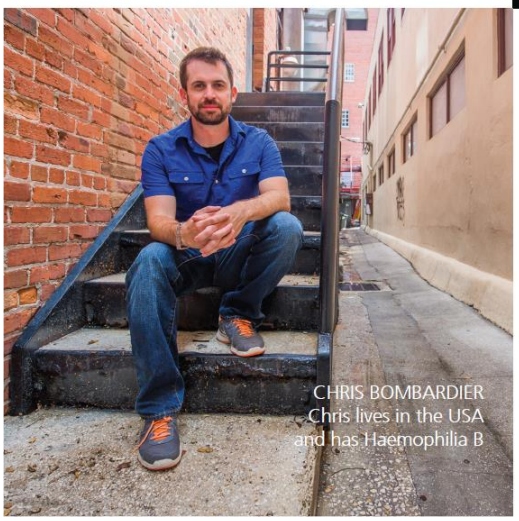
Inspiring Change in Haemophilia – a social media film series from Novo Nordisk

As part of our ongoing commitment to drive change in haemophilia, we recently launched a series of short films – Inspiring Change in Haemophilia – across our social media platforms.

Created in collaboration with some truly inspiring people within the rare bleeding disorder community, each film features a personal story of positive change to raise awareness of key challenges of living with haemophilia.

Our haemophilia-specific videos explore exercise, nutrition and mental well-being, all whilst sharing the patients unique perspectives and advice.

Supporting each film release we share exclusive behind the scenes footage, interviews and practical tips across Facebook, Twitter, Instagram and LinkedIn.



Follow Novo Nordisk to explore our Inspiring Change in Haemophilia films:

@novonordisk

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Novo Nordisk

Or watch the films here under Changing Haemophilia™:
http://video.novonordisk.com

HQMMA/CH/1016/0183. Date of preparation: November 2016

novo nordisk®

Partnering to Change the World for People with Haemophilia

EHC Newsletter 2016

At Pfizer Haemophilia, we're proud of our heritage of innovation through collaboration and partnership in haemophilia care. Through working with the haemophilia community and as part of our ongoing commitment to the field, Pfizer supports adoption of a **"total health"** approach to care – incorporating exercise, physical rehabilitation and patient / physician support tools to optimise patient outcomes. With the patient at the centre of everything we do, Pfizer Haemophilia is supporting expansion of established programmes and the launch of exciting new initiatives for the haemophilia community globally to promote a total health approach to care. In 2016, the following initiatives were developed with the aim of optimising care for people with haemophilia:

Haemoassist® 2 Patient App*

The award-winning **Haemoassist® 2** patient app (smartphone app and website) is designed as an advanced alternative to paper-based diaries, enabling reporting and documentation of bleeds and factor infusions at the touch of a button.[1] In 2016, Pfizer Haemophilia funded the launch of the Haemoassist® 2 tool in new countries across Europe, with the system now available in Germany, Spain and Austria.

The Haemoassist® 2 tool supports people with haemophilia regardless of their choice of clotting factor concentrate. The tool enables patients to manage their treatment proactively within day-to-day life and respond in real-time to joint bleeds, and aims to support interactions between healthcare professionals and patients.

Miles for Haemophilia: Your Personal Best**

In partnership with Alex Dowsett, one of Britain's most talented cyclists who lives with severe haemophilia, the **Miles for Haemophilia: Your Personal Best** campaign encourages the global haemophilia community to challenge themselves to achieve their personal best in whatever way they can: cycling, swimming, running, skipping, walking, jumping, climbing – any activity that keeps you moving.

Pfizer Haemophilia continued to support the programme throughout 2016, as well as launching the exciting initiative **"Haemophilia Dares Me"** where we ask everyone to participate in a Personal Best Challenge and share it on social media as a pledge towards your local Miles for Haemophilia campaign. The campaign aims to increase awareness of the importance of exercise for people living with haemophilia as part of the total health approach to care, and to keep the haemophilia community active.

If you would like to know more or are interested in participating in the programme, please visit the Miles for Haemophilia website www.milesforhaemophilia.co.uk

Physiotherapy School for Haemophilia

The **Physiotherapy School for Haemophilia** promotes the role of physiotherapy for the optimal management of joint health in people with haemophilia. The programme, which partners with Dr Sébastien Lobet (Cliniques Universitaires Saint-Luc, Belgium) and Dr Benjamin Hidalgo (Université Catholique de Louvain, Belgium), aims to provide physiotherapists with hands-on orthopaedic manual therapy training and tools to support the care of people with haemophilia through physiotherapy.

In 2016, we celebrated the launch of the Physiotherapy School for Haemophilia and we hope the continuation of support for the programme in 2017 will help to improve multidisciplinary care and ultimately improve quality of life for people with haemophilia across the globe.

PP-GIP-EUR-0607
Date of preparation: January 2017

These programmes are initiated and funded by Pfizer

Pfizer Haemophilia

Partnering to Change the World for People with Haemophilia

Pfizer Ultrasound Programme

Point-of-care ultrasound is a technique for routine clinical assessment of joints in people with haemophilia that can be used by non-imaging haemophilia specialists to detect joint effusion, synovial hypertrophy and abnormalities involving osteochondral surfaces.[2,3]

Pfizer is committed to advancing innovation and imaging in haemophilia care. Partnering with Carlo Martinoli (Professor of Radiology, University of Genoa, Italy) and haemophilia experts, Pfizer have pioneered this technique for the haemophilia community with the aim of optimising joint health within the total health approach to care. The ongoing commitment of Pfizer to the ultrasound programme aims to expand our knowledge and understanding of the value of point-of-care ultrasound, and dissemination of technical expertise to healthcare professionals in order to bring this innovation in imaging direct to patients.

At Pfizer Haemophilia, we understand the value of collaboration and partnership. Through collaboration with advocacy groups, such as the European Haemophilia Consortium (EHC) and the World Federation of Hemophilia (WFH), we aim to work together with key partners in the haemophilia community to best meet the spectrum of needs.

EHC Website and Learning Portal

Part of the EHC's work is to educate their member organisations through close collaboration to ensure a strong collective voice for people with rare bleeding disorders.[4] Pfizer Haemophilia understands the importance of disseminating this information, and therefore supported the recent update and revision of the **EHC Website** in 2016 to enhance this valuable resource. Pfizer Haemophilia provided further commitment to patient advocacy groups by providing an educational grant for the development of the EHC Learning Portal. The **EHC Learning Portal** is an online tool, designed to support the dissemination of education around principles of health technology assessment and health economics. The tool has been designed as an interactive resource to allow for self-directed learning at a pace that suits the user.

We hope our support for the education of EHC members will further strengthen the patient voice within the haemophilia community and the improvement of care for the future.

WFH Twinning Programme

Pfizer Haemophilia is proud to be the exclusive sponsor of the **WFH Twinning Programme**, encouraging exchange of experience, skills and resources related to haemophilia treatment between countries. In 2016, Pfizer Haemophilia celebrated the 15-year anniversary of the programme's launch, during which time 212 partnerships across 111 countries have been established.[5]

We are proud to be making a difference by enhancing best practices and treatment access to better the lives of those living with a bleeding disorder.

If you would like more information on these programmes, please contact your local Pfizer representative. We look forward to continuing to support the community through these important collaborations and partnerships so that we can work together to ***Change the World for People with Haemophilia***.

References: 1. StatConsult, Haemoassist® 2: www.statconsult.de/de/haemoassist_europrise (Accessed January 2017); 2. Martinoli C, et al. *Thromb Haemostas* 2013;109:1–10; 3. Colvin B, et al. *Eur J Haematol* 2013;90(Suppl. 73):1–9; 4. EHC website: Mission and Objectives. www.ehc.eu/about-ehc/mission-and-objectives/ (Accessed January 2017); 5. WFH Twinning Programme: www.wfh.org/en/twins (Accessed January 2017).

*StatConsult is the legal manufacturer of Haemoassist® 2 and Pfizer hold sole distribution rights. Haemoassist® 2 is a CE-marked medical device in Europe, currently available in Germany, Spain and Austria. It is currently neither registered nor available in other regions.

**The Miles for Haemophilia: Your Personal Best campaign is initiated and funded by Pfizer.

These programmes are initiated and funded by Pfizer Pfizer Haemophilia

We are Roche

Founded in 1896, the Roche Group is a global pioneer in pharmaceuticals and diagnostics and is committed to advancing science to improve people's lives. Headquartered in Basel, Switzerland, Roche is active in over 100 countries and in 2015 employed more than 91,700 people worldwide. The company is strongly committed to patient focused scientific innovation and in 2015, invested CHF 9.3 billion in Research & Development.

We develop innovative medicines and diagnostic tests that help millions of patients globally for conditions of significant unmet need. Our combined strengths of pharmaceuticals and diagnostics under one roof have made Roche the leader in personalised healthcare – a strategy that aims to fit the right treatment to each patient in the best way possible.

With more than 140 medicines on the market and thirty medicines included in the World Health Organization Model Lists of Essential Medicines, we are proud to be serving patients around the world. Our medicines are indicated for a broad range of disease areas[8] including:

- Oncology
- Haematology
- Haemophilia[9]
- Neuroscience
- Infectious diseases
- Immunology
- Cardiovascular and metabolic disorders
- Ophthalmology

Roche's commitment

The Roche Group's involvement in haemophilia-related research stretches back several decades. In 1984 research scientists who were members of the Department of Molecular Biology and the Protein Biochemistry unit at Genentech (a member of the Roche Group) published ground-breaking research related to the structure and genetics of human Factor VIII as well as the expression of Factor VIII from recombinant DNA in the scientific journal *Nature*[10].

For more than 20 years, Roche has been innovating and delivering medicines for people with various blood disorders. We continue to invest significantly to bring innovative treatment options for these conditions, including haemophilia A, and are committed to developing novel approaches to advance the management of these serious conditions. Roche is dedicated to developing novel monoclonal antibodies (mAbs) in haematology beyond oncology, including the development of the investigational haemophilia A treatment.

Our commitment to patients is demonstrated in many ways throughout the company and is fundamental to what we believe to be Roche's most significant contribution to society: to create, produce and deliver innovative solutions of high quality for unmet medical needs. Our shared commitment to patients makes us eager to listen, understand and collaborate with the haemophilia community to improve patients' access to innovative treatments and services. At Roche we are convinced that an open dialogue and transparent exchange of information among

8 Roche internet www.roche.com Accessed 25 October, 2016

9 Investigational indication

10 1-3 [Vehar et al, Nature 1984/p337; Gitschier et al, Nature, 1984/p326; Wood et al, Nature, 1984/p330]

all partners in the healthcare community is vital to advancing access and healthcare delivery to patients. Building and sustaining relationships with patient groups is an important way for us to emphasize our commitment to patients and to society as a whole. For more information on Roche in haemophilia, please visit www.roche.com/haemophilia

Our Inaugural Presence at the EHC Annual Conference 2016

For the first time, this year, Roche had the privilege to participate in the EHC Annual Conference in Stavanger, Norway. It was a unique experience and an opportunity for our team to meet and engage with members of the EHC community in interesting and insightful conversations, listen and share a common vision for the future of haemophilia.

Through an interactive corporate booth, we were able to meet and engage with participants of the conference. By asking attendees to answer the question "*If you could rewrite the future of haemophilia A, what would it look like*?" we were able to gain insights and initiate dialogue, receiving 46 answers in total. These were written on "*postcards*" that were stuck on the booth walls. Feedback from patients and HCPs covered subjects such as access to treatment, disease management and the development of new treatments.

As part of our presence at the conference, we asked participants -"***If you could rewrite the future of haemophilia A, what would it look like?"***

We were pleased that so many shared their ideas with us, which help us to learn and understand our common aspirations. We received around 50 written answers from the participants which covered subjects such as access to treatment, disease management and the development of new treatments:

"I would like to be able to tell my children "Yes, I can play with you today."

"Access to new medicines for all patients with haemophilia A across the world"

"*Solidary patient community that shares its best practice and supports advancement in treatment for all rare diseases".*

Thank you for sharing your insights and providing us with valuable feedback!

Roche is proud to support and work in partnership with the haemophilia community in order to address the existing challenges. Haemophilia is a new disease area to our company and thus, we are eager to learn from the community to be able to put that innovation to work in the most powerful, meaningful way. Key principles of our work with patient groups are integrity, transparency, trust and mutual respect. We enter into this new relationship with the haemophilia community and are committed to sustaining a long-term working relationship.

We look forward to continuing our collaboration with EHC in 2017 and the years to come!

Unmet needs require unmatched commitment.

Our purpose is clear: to make a difference in the lives of those living with and affected by rare diseases and highly specialized conditions.

For more information, please visit shire.com

Shire

Third EHC Youth Debate: An evolving format to prepare young leaders for the future through opposition research

Wills Hughes-Wilson, Senior Vice President, Chief Patient Access Officer & Phil Wood, Vice President, Global Commercial Lead, Haemophilia, Sobi

Securing the future of an organisation is arguably just as important as delivering on its current mission and content. Forward-looking companies and organisations devote a substantial amount of time to training, development and succession-planning, to secure they are able to be sustainably successful.

In recent years, the European Haemophilia Consortium (EHC) has been focussing attention and programmes on developing its youth leadership, with the objective of securing that the current leaders pass on their experience to prepare the future of the organisation. One way that Sobi aims to support these development activities is by donating its company symposium during the annual EHC Conference back to the EHC, to create a forum where young EHC members can practice debating and advocacy skills together senior EHC leadership. This happened again for the third time this year on 7th October 2016 at the Annual Conference in Stavanger, Norway.

The format remained the same – with young leaders debating against established clinicians; and the clinicians being asked to argue the opposite of their own points of view. This year, however, the content has evolved and the forum touched on important and serious subjects – at times uncomfortable. *When and where is it OK to ask or share private health-related data? Who decides? Why is it important?*

The programme included debates on three topics of importance to the communities: *"Adults need prophylaxis vs. Adults don't need prophylaxis;" "We don't spend enough time on inhibitor patients vs. We spend too much time on inhibitor patients who are a small percentage of our community;"* and *"Access to hepatitis C treatment should be a priority for NMOs vs. It should not be a priority for NMOs."*

The format is intended to stay fun and engaging – which Brian O'Mahony, EHC President, reminded the

Pannellists during the EHC Youth Debates, from left to right: Mr Stephan Meijer, Prof Cedric Hermans, Mr Stefan Tasic, Mr Brian O'Mahony, Prof Mike Makris and Mr Cristian Ungureanu

audience of as he opened the session in his role as Chair: "*This is not a recombinant, this session is a blood sport!"*

Stefan Tasic from Serbia presented a well-constructed, evidence-led argument about the importance of prophylaxis. Prof Cedric Hermans, President of the European Association for Haemophilia and Allied Disorders (EAHAD), taking the opposing view, countered by stating that, while prophylaxis reduces joint bleeds, it does not preserve joints and that alternative therapies could be equally beneficial. The vote showed that the audience were not convinced, voting in favour of prophylaxis for adults as well as children. In the second debate, Cristian Ungureanu from Romania made the case for spending more time on patients with inhibitors by highlighting learnings from real-life experiences. Even though Professor Paul Giangrande, member of the EHC's Medical Advisory Group, claimed in his reply that too much time is spent on the very small percentage of inhibitor patients, and that clinical outcomes for people with inhibitors have much improved in recent times, he could not convince the room. Not least because, as one audience member pointed out, stating that five per cent "*doesn't count*" doesn't get traction when you are addressing a room full of people with a rare disease. The observation raised laughter and applause. Finally, making a strong

case that access to hepatitis C treatment should be a priority for National Member Organisations (NMOs) was Stephan Meijer from the Netherlands. He made it tough for Prof Mike Makris, also a member of the EHC's Medical Advisory Group, to defend his "*view*" that, while treatment is indeed obvious, cost remains a barrier.

All three of the youth presenters were passionate, professional and persuasive. Their senior leadership counterparts were generous with their time and their engagement. In the end, the audience voting said it all – the young presenters won their arguments. The debate, while conducted in a fun format, drew thought-provoking comments and insightful reactions from the audience, because at the heart of each presentation were tough, real-life questions facing the haemophilia community right now.

Prof Paul Giangrande took part in the Youth Debates

In thanking the youth leaders for making their cases so well; and the senior leadership for their *"performance,"* Brian took the opportunity to emphasise key learnings and to highlight that such debates are part of an important initiative with a specific purpose: to teach and practice opposition research and to understand its importance when making a case as an advocate. Entertainment aside, the debates help to illustrate a serious point: if you are going to go to your government to make a case for increased resources, for example, this kind of preparation to anticipate potential opposition and how to prepare for it is an extremely useful technique to master.

Cultivating a strong youth leadership by involving its young people in this way is a key strategy to build sustainability, helping to secure the future of EHC's leadership on behalf of the communities it represents. Younger members who take an interest and step up and lead today are the ones who equip themselves to lead effectively tomorrow.

Brian also highlighted that, when the EHC youth engage and actively participate like this – passionately practicing an advocacy role in such a forum – we can be assured that people with haemophilia and their families will be truly represented in the future, giving the community a voice and, thus, be more likely to have an influence over the future of their treatment choices.

It is also clear that young members of the EHC already have a lot to bring to the community. They relate to, and have unique insights into the potential impacts of decisions concerning young people, instinctively knowing how to effectively connect and communicate key issues. For this reason Sobi supports the EHC's development initiatives.

Mr Brian O'Mahony chaired the Youth Debates

Sometimes it is hard to know if a format will work or if it will continue to work. Sometimes the best thing to do is to give it a try. Three years into the Youth Debates, the feedback from participants indicates this is a valuable forum for debate and development. There are many topics of importance to the community that deserve examination in a collaborative, community-led setting. Indeed, there have been calls from participants for the session to be expanded to provide a platform for increasingly real debate forums. As the EHC continues to build recommendations and policies aimed at continually improving diagnosis, treatment and care for its members and the communities they represent, including the updating of the recommendations from the European Directorate for Quality of Medicines and Healthcare (EDQM), it looks like there will be no shortage of topics.

HEMOPHILIA LEAD

BAYER'S COMMITMENT TO UNITING, EDUCATING AND EMPOWERING HEMOPHILIA PATIENT ADVOCATES

HEMOPHILIA ADVOCACY ADVISORS BOARD


Val Bias
National Hemophilia Foundation


Carlos Gaitán Fitch
Federation of Hemophilia of Mexico


Dr. Yasuharu Nishida
National Hemophilia Network of Japan


Brian O'Mahony
Irish Haemophilia Society


Dr. Uwe Schlenkrich
German Haemophilia Society


Mark Skinner
World Federation of Hemophilia


Pam Wilton
Canadian Hemophilia Society

Patient advocates have unique and direct insight into the needs and challenges of the hemophilia community. They often serve as their voice on key issues, so it is critical for them to have the tools and experience to effectively advocate for their constituents. To help support the efforts of this important community, Bayer offers a long line of advocacy support, programs and partnerships to help patient advocates discuss the issues they face and empower them to affect change.

In 2011, the international Hemophilia Advocacy Advisors Board (HAAB) was established by Bayer to help identify unmet advocacy needs for hemophilia patients and create programs that would galvanize advocates and help them drive impact in their communities. To address the need of educating and uniting patient advocates, the HAAB created the Global Haemophilia Advocacy Leadership Summit, a gathering of top hemophilia advocates from across the globe, to work to solve issues critical to the advocacy community.

Since its inauguration in 2012, the HAAB has hosted a Summit every year in cities around the world. The most recent event took place in January 2016 in Lisbon, Portugal and attracted 28 advocates from 21 countries. In 2017, we aim to explore the topic of health economics and the skills needed to become a leader in the hemophilia advocacy community.

HEMOPHILIA LEAD PROGRAMS

HEMOPHILIA ADVOCACY ADVISORS BOARD (HAAB) is a group of leading patient advocates dedicated to identifying unmet needs in the bleeding disorders community. Members of the HAAB also act as counselors to the HEMOPHILIA LEAD programs.

PARENTS EMPOWERING PARENTS (PEP) facilitates dialogue and information sharing among parents of children with hemophilia. PEP equips parents with the skills and knowledge needed to respond to the needs of their child with hemophilia. www.pepprogram.org

STEP UP REACH OUT (SURO) is a youth leadership program designed to develop and train young men ages 21-25 in the bleeding disorders community. In addition to the global program, it also has a Latin American chapter and plans to expand in other regions across the globe. www.hemophilialead.net

ADULT FELLOWSHIP FOR INTEGRATING RESPONSIBLE MENTORS (AFFIRM) is a fellowship program designed to mentor and help men ages 26-38 with hemophilia develop and grow their leadership skills. www.hemophilialead.net

GLOBAL HAEMOPHILIA ADVOCACY LEADERSHIP SUMMIT convenes global advocates to discuss important issues affecting the hemophilia community and provides the training to implement change.

THE INTERNATIONAL HEMOPHILIA ACCESS STRATEGY COUNCIL (IHASC) is a group of world-renowned access experts, patient advocates and health economists who work with Bayer to provide solutions for approaching today's changing access landscape.

G.MKT.SM.HEM.11.2016.1201

Hemophilia Solutions
Never Losing Sight of the Human Factor

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

CSL Behring is markings its 100th anniversary

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

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

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

Prof Holmes chaired the CSL Behring symposium at the EHC Annual Conference

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

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

Changing the Conversation

inhibitors in hemophilia A

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

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

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

How prevalent are inhibitors?

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

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

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

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

Learn more about inhibitors and recent study results

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

1. Summaries of up-to-date inhibitor data and study results
2. Videos of leading hematologists discussing actual topics of interest

Visit InhibitorInfo.com

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

GRIFOLS

Calendar of Events

EHC Events

Mar 7:	Round Table of Stakeholders on Clinical Trials *Brussels, Belgium*
Apr 7-9:	Youth Leadership Workshop *Amsterdam, the Netherlands*
Apr 19:	World Haemophilia Day event *Strasbourg, France*
Jun 27:	Round Table of Stakeholders on Orthopaedic Aspects in Haemophilia Care *Brussels, Belgium*
Jun 29-Jul 2:	Leadership Conference Location *TBC*
Sep 15-17:	Workshop on Tenders and Procurement Location *TBC*
Oct 6:	Annual General Assembly *Vilnius, Lithuania*
Oct 6-8:	Annual Conference *Vilnius, Lithuania*
Nov 17-19:	Workshop on New Technologies in Haemophilia Care Location *TBC*
Nov 28:	Round Table of Stakeholders on Extended Half-Life Coagulation Factors Use and Measurements *Brussels, Belgium*
Nov 30-Dec 3:	Inhibitor Summit Location *TBC*

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

Other events:

2017

Feb 1-3:	10th Annual Conference of the European Association for Haemophilia and Allied Disorders (EAHAD) *Paris, France* – More information at http://eahad2017.com/
Mar 3-5:	7th International Symposium on Women's Health Issues in Thrombosis and Haemostasis *Barcelona, Spain* - More information at http://www.whith.org/

Announcements

Invitation to contribute: Evaluation of chronic pain and adherence to treatment among the European population with haemophilia

Dear readers,

Herewith we would like to announce a European-wide study to investigate chronic pain and adherence to prophylactic treatment in patients with haemophilia and we invite your National Member Organisation (NMO) to participate.

The objective of this study is to explore potential factors that influence adherence to prophylactic treatment among the European haemophilia patient population using a self-report questionnaire. Identifying incentives and barriers to treatment adherence would allow to specifically address these issues in patient communication and to develop tailored interventions to support adherence.

Background

Adherence to treatment is essential in haemophilia due to the need for lifelong replacement therapy of clotting factor concentrates, especially in prophylaxis. As most patients with haemophilia are introduced to home treatment, patients have to accept the need for therapy and comply with a recommended infusion schedule. Adherence is increasingly recognised as an important factor for health-related quality of life and psychosocial lifestyle. It has been demonstrated that the clinical outcome improves if patients are adherent to treatment[11]. On the other hand, reduced adherence can worsen haemophilic arthropathy and chronic pain.

We are looking for 800 to 1,000 patients from all age groups with moderate or severe haemophilia from different European countries to respond to a questionnaire anonymously.

To evaluate the adherence to treatment the questionnaire VERITAS Pro (Validated Haemophilia Regimen Treatment adherence Scale for Prophylaxis) has been developed and validated by Duncan et al. from the Indiana Hemophilia and Thrombosis Center (2010)[12]. It consists of six sub-scales that examine whether or not the patient infused at the recommended time, used the recommended dose, planned ahead to have adequate factor and supplies on hand, remembered to infuse, skipped infusions and communicated with the haemophilia centre. Each item is quantified on a five-point scale ranging from “Always” to “Never." Potential scores range from 24 to 120 with a lower score indicating increased adherence. For studying the chronic pain levels we integrated the revised Faces Pain Scale (FPS-R)[13] in the questionnaire. The FPS-R is a visual scale composed of six faces describing pain intensity. To identify potential impact factors additional data should be captured as sociodemographic data, severity of haemophilia, treatment characteristics (including history of switching or using of longer-acting factor concentrates), clinical variables, modes of medical care and health outcomes. For analysis the VERITAS-Pro total scores, subscale scores and health outcomes will be compared between the different age and population groups, pain levels and the demographics.

11Krishnan S, Vietri J, Furlan R, Duncan N. Adherence to prophylaxis is associated with better outcomes in moderate and severe haemophilia: results of a patient survey. Haemophilia 2015; 21(1):64-70.

12Duncan N, Kronenberger W, Uncan N, Roberson C, Shapiro A. VERITAS- Pro: a new measure of adherence to prophylactic regimens in haemophilia. Haemophilia, 2010, 16: 247–255.

13The Faces Pain Scale – Revised, FPS-R. International Association for the Study of Pain (IASP) ©2001.

In our recently published German survey[14] (see EHC-Newsletter December 2015) we already analysed answers from nearly 400 haemophilia patients with regular prophylaxis according to four age groups: 0-14, 15-19, 20-59 and ≥ 60 years of age. The mean total VERITAS-Pro score for the whole sample was 37.3±11.7 (range 24 to 94), well below the defined 57-point-cut-off for non-adherence. Adherence was highest in the youngest patient group < 14 years; 100 per cent of patients up to 19 years were adherent, possibly attributed to a parental control of infusion schedules. We could find several age-specific impact factors influencing adherence to treatment. Patients with severe haemophilia were more adherent than patients with moderate haemophilia in all age groups except for the adolescents (15-19 years). Receiving care in a haemophilia treatment centre significantly improved adherence at least in the middle-aged patients. Limited group sizes did not allow significant observations for the other age groups. Future studies with higher patient numbers have to be conducted to confirm the influence of further age-specific parameters on adherence to prophylactic treatment.

This international study is supported by a HERO research grant from NovoNordisk. It is the first attempt to assess adherence to treatment among paediatric and adult patients in Europe. Considering the rarity of the disease and the small patient population, the collaboration of several patients' organisations is of great importance and highly appreciated.

Best regards,

Wolfgang Miesbach and Werner Kalnins

Dr Wolfgang Miesbach is a professor of Internal Medicine/Haemostaseology and Director of the Haemophilia Centre at Goethe University Hospital in Frankfurt/Main. Werner Kalnins was the President of the Deutsche Hämophilie-Gesellschaft (DHG), the German National Member Organisation, from 2004-2016. They recently published an article about adherence to prophylactic treatment in haemophilia patients in Germany. Here they announce the subsequent European-wide survey.

If your NMO is interested in participating, please contact the German Haemophilia Society (Tel.: +49/40/6722970, e-Mail: dhg@dhg.de) or Dr Miesbach (Tel.: +49/69/63015051, e-Mail: wolfgang.miesbach@kgu.de).

Authorship for publication will be guaranteed. In case your NMO needs financial assistance for conducting the survey, a reimbursement of max. 10€/participant is possible.

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Sexuality and Bleeding Disorders

By Jo Eerens, EHC Membership Officer

Members of the Van Creveldkliniek, Netherlands, published a third book in the series '*Haemophilia Care and Treatment: Sexuality and Bleeding Disorders*'. It is a remarkable and successful attempt to break a taboo on this delicate matter.

This book is written first of all for medical caregivers: doctors, nurses, physiotherapists, psychologists, social workers in the field of haemophilia and other bleeding disorders.

14Miesbach W, Kalnins W. Adherence to prophylactic treatment in patients with haemophilia in Germany. Haemophilia 2016, in press.

The book opens by acknowledging that bleeding disorders affect the sexuality and sexual behaviour of the patient. The following chapters approach this topic for different age groups. Other topics coverd by the book include: acute bleeds, women who are von Willebrand patients, arthropathy, infectious diseases (HIV and hepatitis C) and the psychological impact on the sexuality of a patient with a bleeding disorder. Finally, the last chapter provides practical advice for the comprehensive care centre on how to start a conversation with the patient on their experience with sexuality. In all the chapters you can read patient testimonials on the different subjects.

This book is meant to help health care professionals approach the topic with their patients. It is meant as a tool to break away from taboos and openly discuss about sexuality and bleeding disorders by providing very practical information on issues patients may be facing. The book is also meant to raise awareness with caregivers regarding the issues faced by patients with regard to sexuality.

Although most of the topics are discussed in a *'medical'* way, a well-informed haemophilia patient can easily read the information provided in the book. Also I was very pleased to see that women and von Willebrand patients were always included in the topics. There is, in fact, a full chapter devoted to "*Sexuality in women with von Willebrand Disease."* It is refreshing to see that this topic is not only approached from a male's perspective because there is also so much to say about the impact of a bleeding disorder for a female patient. Some may perhaps expect from this type of book a more in-depth analysis of the impact of the disease on sexuality. However, this book is a preparatory read and meant to inform to the conversations between health care professionals and patients.

I mostly congratulate the team of the van Creveldkliniek for this publication. My hope is that caregivers in other countries and haemophilia centres will take the opportunity to break the taboo and give adequate support to patients facing challenges in this area. I would say that, it is a '*must read'* and *'must have'* on the bookshelves of haemophilia centres across Europe.

'Sexuality and Bleeding Disorders' editors: Eveline Mauser-Bunschoten, Woet Gianotten, Lily Heijnen, Annemarie de Knecht-van Eekelen, Utrecht – van Creveldkliniek – 2014. (Please write to Dr. Eveline Mauser-Bunschoten for more information on how to get the publication).