# EHC Newsletter December 2015

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Editor: Amanda Bok
Editorial Committee: Jo Eerens, Radoslaw Kaczmarek, Brian O’Mahony, Olivia Romero-Lux, Laura Savini
AISBL EHC registered office: rue de l’Industrie 10, 1000 Brussels, Belgium
Tel. +32-2-893-24-70 - Email office@ehc.eu
No. 887.106.966
President and CEO Report

Extended edition

For the past few years our end-of-year message has focused on the EHC’s continued growth, which is thankfully evidenced again this year – and not least by the size of this newsletter – and for which we are grateful to our external partners and volunteers for continuing to make our work – and dare we hope our positive impact in Europe – possible.

The highlights of the last quarter

It has been a particularly busy few months since our August edition – and beyond that another productive and enriching year. This year we held three successful Round Tables; the last one, which was held in the wake of the Brussels security shut-down, still brought together more than 30 avid participants to discuss the unique challenges of mild and moderate haemophilia (see pg 35). We successfully and proudly piloted the EHC Leadership Conference (see pgs 22 and 24) and followed it immediately with our annual EHC Conference (see pg 13), which proved to have the highest attendance of our conferences to-date. We trained our Russian-speaking National Member Organisations (NMO) in economic concepts (see pg 29) and our wider stakeholders in new technologies (see pg 30). We were delighted to launch our European Inhibitor Network earlier in the year and held our first event during our Conference in Belgrade in October (see pg 17). During the same conference, we also started a conversation about the activities that are conducted throughout Europe to serve the women of our rare bleeding disorders community (see pg 19). During the same conference, we also started a conversation about the activities that are conducted throughout Europe to serve the women of our rare bleeding disorders community (see pg 19). We conducted our joint advocacy visit with the European Association for Haemophilia and Allied Disorders (EAHAD) and the World Federation of Hemophilia (WFH) in Latvia and Estonia (see pgs 55 and 57). We launched our third ‘Haemophilia Care in Europe’ survey and, last but certainly not least, we worked with the talented and internationally acclaimed director/producer Goran Kapetanovic on a documentary that movingly captures the human dimension behind haemophilia (pg 63) – more on that project to come in 2016.

Elections and appointments

We were pleased to co-opt Michael van der Linde from the Dutch NMO unto the Steering Committee and see the appointments of Ivan Šebest and Aleksandra Ilijin from the Slovakian and Serbian NMOs, respectively, to the Youth Committee (see pg 36). Equally, we are pleased to announce the formation of our Inhibitor Working Group and welcome onto it new volunteers Mirko Jokic, Hannele Kareranta, Dr Oleksandra Stasyshin, Christina Burgess, Carl Fredrik Gustafsson and Elisabeth Olesen from the Serbian, Finnish, Ukrainian, UK, Swedish and Danish NMOs, respectively, who will work alongside ongoing volunteer Miguel Crato, President of the Portuguese NMO, and our Steering Committee and Medical Advisory Group members to roll out the inhibitor programme over the next few years. Finally, at the Annual Conference in Belgrade, outgoing President Brian O Mahoney was re-elected as President for the next four years.

Staff changes

We are pleased to announce some staff changes for 2016. In the first part of the New Year, long-standing office administrator Jo Eerens will gradually transition into the new position of Membership...
Officer, a new office support will be hired to take over his previous duties, and our shared EHC-EAHAD project consultant, Aislin Ryan, will move full-time to EAHAD where she has transitioned into a newly created Managing Director role.

Looking ahead

2015 has been another enriching year and we intend for 2016 will follow in a similar manner, with many if not most of our current activities to become ‘mainstays’ of the EHC’s work in Europe. In addition, 2016 will see new developments and a shifting landscape in treatment and care, not least with the European Commission’s call for applications for European Reference Networks (ERNs) but also with the introduction into the European market of extended half-life products, expanded hepatitis C treatments and other novel technologies coming down the pipeline as well as a further meeting of the European Directorate for the Quality of Medicines and Healthcare (EDQM), an organisation part of the Council of Europe, which should lead to the promulgation of new recommendations on haemophilia. It remains an exciting period in haemophilia.

Many thanks, happy reading and happy holidays!

For now, however, we look forward to the winter break and take this opportunity to sincerely thank all of our external partners and volunteers for your support, time and dedication to the EHC. We leave you with this end-of-year newsletter, in which we hope you will find interesting, compelling and informative articles, and wish you happy reading – and happy holidays.
EHC News

Comprehensive care in haemophilia: Physiotherapy and OB-GYN

In this issue, we continue our series on comprehensive care in haemophilia by looking at physiotherapy and at specialised gynaecology and obstetrics for women with rare bleeding disorders and carriers. Laura Savini took advantage of the EHC Annual Conference (see page 13) to interview two medical specialists from each of these disciplines speaking during the event.

Physiotherapy in the comprehensive care of haemophilia

Dr Sébastien Lobet* interviewed by Laura Savini**

Dr Sébastien Lobet works at the Cliniques Universitaires Saint Luc in Brussels, Belgium; alongside Prof Cédric Hermans, Vice-President of the European Association for Haemophilia and Allied Bleeding Disorders (EAHAD). Sébastien tells us more about being a physiotherapist and working with people with rare bleeding disorders.

On physiotherapy

Physiotherapy is a technique to restore musculoskeletal health by giving back motion and muscle strength to patients. It is generally performed with hands although the physiotherapist can also use a medical device or a machine. People with haemophilia (and other rare bleeding disorders impacting joints) suffer from joint problems and muscle weakness and therefore it is important to use physiotherapy to restore their strength and mobility.

On the role of the physiotherapist

Sébastien currently has two jobs, he is both a physiotherapist and a researcher. As such, he fulfils four different tasks in the haemophilia centre where he works.

His first role is to be a treater for people with haemophilia, for example, Sébastien will provide care for people with haemophilia following orthopaedic surgery or following an acute bleed. Another one of Sébastien’s responsibilities is to assess joint health in both children and adult patients. In children, his aim will be to preserve good joint status and to detect the first signs of arthropathy. In adults he will try to estimate whether joint health is improving or becoming impaired and try to correct this. Sébastien also manages the musculoskeletal condition of his patients. This requires, for example, working closely with other specialists such as haematologists, orthopaedic surgeons and the rehabilitation department. It is important for physiotherapists to ensure that specialists are well informed of the musculoskeletal condition of the patient.

Finally Sébastien also conducts clinical research in the field of biomechanics. His main research area at the moment is the development of new tools and methods to assess joint health more precisely and to understand the impact and consequences of joint arthropathy on an individual’s gait and balance and on his or her ability to conduct daily activities. The research is conducted with both the Université Catholique de Louvain (UCL) and the Katholiek Universiteit Leuven (KUL) in Belgium. Although this research is designed for and carried out on people with haemophilia, it will be adaptable to patients...
suffering from joint arthropathy resulting from other conditions such as osteoarthritis, juvenile idiopathic arthritis, rheumatoid arthritis and for people suffering from foot problems in diabetes.

**On working with other healthcare professionals**

Besides working closely with orthopaedic surgeons and haematologists as mentioned above, Sébastien also works closely with nurses and paediatricians. Moreover, Sébastien is in close contact with other physiotherapists who do not regularly treat people with haemophilia. Belgium has many good physiotherapists and patients are lucky enough that they can choose to see a physiotherapist that lives close by instead of travelling to the haemophilia centre for each consultation. Therefore, one of Sébastien’s jobs is to be the liaison person between the centre and the physiotherapists who may have never seen a patient with haemophilia. Sébastien will explain haemophilia to these non-specialist physiotherapists and provide details on the orthopaedic treatment that the patient has received (e.g. a new prosthesis).

**On preventing joint problems**

The key element to preventing problems in joints is to have access to prophylactic treatment with coagulation factor concentrates. Sébastien notes great differences between his younger and older patients, with younger patients who have been on prophylaxis since birth having much better joint protection compared to older generations who had not received good treatment in their youth. Furthermore, when patients have access to prophylaxis, regular physical activity is recommended while for patients with limited access to treatment physical activity must be performed with caution as it may put them at risk for joint bleeds.

Another important problem that affects joints is obesity. This is not only because of the additional load put on the joints by extra weight but also because fat tissue acts as an endocrine organ releasing inflammation in the whole body. Therefore a big role of the physiotherapist is to educate both children and adults on the risks associated with obesity, to try to prevent obesity in both children and adults, and to reduce obesity in affected patients.

The last issue that impacts joint health is the willingness of people with haemophilia to take the time to care for themselves. In theory a person suffering from severe haemophilia should see a physiotherapist at least two to three times a week for preventive or curative physiotherapy sessions. Unfortunately, this is not always possible as people with haemophilia generally lead very busy lives. It is therefore important for the physiotherapist to talk to his patients and stress the importance of taking the time to do regular physiotherapy to preserve and improve joint health.

**On European professional collaboration**

Sébastien is a member of the Physiotherapists Committee of the European Association for Haemophilia and Allied Disorders (EAHAD), a European organisation representing healthcare professionals working in the area of haemophilia and other rare bleeding disorders. For Sébastien, it is important to have a platform for European collaboration to be able to exchange with peers who are faced with the same types of patients and issues in order to exchange and learn from one another.

The EAHAD physiotherapy group hopes, through joint collaboration, to improve its members’ professional knowledge, to establish and improve physiotherapy guidelines, to develop a network for physiotherapists and to pinpoint the current status of physiotherapy practice in haemophilia care in Europe.
On the treatment landscape in Europe

As Sébastien explained access to prophylaxis is not equal in all European countries and, while some regions benefit from good access, in others this is still lacking.

With regard to physiotherapy services, the level of access and the quality of these services throughout Europe remains a big question mark and this is one of the questions that will be explored by the EAHAD Physiotherapy Committee. Sébastien notes that sometimes even in a single country the services available to patients can vary hugely depending on whether the service is provided in a haemophilia centre or if the patient lives in a more remote area. Another thing that will be looked at by the EAHAD group is the level of training of physiotherapists in Europe, because this will also affect the role the physiotherapist play. For instance, it is likely that the higher the level of education of the physiotherapist, the easier the interactions with the medical team in the haemophilia treatment centre will be.

On current and upcoming challenges in physiotherapy for haemophilia

For Sébastien, there are three main challenges in physiotherapy. The first one is the assessment of patients. Thanks to prophylaxis there is a huge improvement in the musculoskeletal health of children and, therefore, it will become increasingly difficult to diagnose and assess arthropathy. For example, although the revision of the haemophilia joint health score (HJHS) has improved this working tool, it is still, in some instances, not sensitive enough to assess the onset of arthropathies. Sébastien also believes that more could be done to prevent joint bleeds.

The next challenge is that although physiotherapy is effective and efficient in patients with haemophilia, it is very difficult to scientifically prove its efficacy and efficiency. This is because physiotherapy encompasses many different techniques and it is sometimes difficult to isolate one technique to prove its effectiveness. Therefore, although not impossible, it is very difficult to conduct clinical trials to prove the efficiency of physiotherapy. In the last five to ten years there has been much research conducted in the field of osteoarthritis that proves the efficiency of manual therapy. According to Sébastien, haemophilia is simply a super osteoarthritis and therefore it could be easy to transfer this knowledge to the field of haemophilia. A challenge for the future would be to develop guidelines and to prove that physiotherapy in haemophilia is efficient and cost-effective compared to factor replacement. Furthermore, physiotherapy has no or very few side-effects. So in Sébastien’s opinion it is very important to use this non-pharmacological method to improve and preserve joint health.

The last challenge is to recognise specific competences of physiotherapists in the haemophilia centre, which as he described encompass more than simply treating patients but also include the assessment of patients, liaising with other healthcare professionals and conducting research. For instance, specialisations for nurses who need specific competences already exist, as in the cases of haemophilia nurses or nurses specialised in diabetes. Sébastien believes that the same should be applied to physiotherapists and this will be one of the objectives of the EAHAD’s Physiotherapy Committee.

On training for physiotherapists

In Sébastien’s opinion, in order to become a specialised physiotherapist for people with rare bleeding disorders and to work in a haemophilia centre, one needs to see a lot of patients. Therefore it is crucial...
that each centre has one or two physiotherapists that solely take care of people with haemophilia. In many cases physiotherapists are attached to the rehabilitation department but in practice the best would be that the same physiotherapists would treat people with haemophilia because there is an important learning curve in dealing with people with haemophilia.

At the moment there is no specific physiotherapy training for haemophilia. However, this will change in January 2016 when he and a colleague will launch the HAE-MOTION-Training course for physiotherapists specialized in haemophilia care.

Physiotherapists looking to learn more about the EAHAD Physiotherapy Committee’s work can send a message to physio@eahad.org

* Dr Sébastien Lobet is a physiotherapist and researcher working at the Cliniques Universitaires Saint Luc in Brussels, Belgium.

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

Gynaecology and Obstetrics in the Comprehensive Care of Haemophilia

Rezan Kadir* interviewed by Laura Savini**

Dr Rezan Abdul-Kadir is a Consultant Gynaecologist with subspeciality in foetal medicine and a special interest in women with bleeding disorders working at the Royal Free Hospital in London, United Kingdom (UK). Rezan tells us more about her work caring for women with bleeding disorders and carriers.

About obstetrics and gynaecology

Obstetrics and gynaecology are all about bleeding. Gynaecology is associated with bleeding, for example, through menstruation, and in obstetrics, a normal amount of bleeding occurs during delivery and miscarriages. If you have a bleeding disorder you are more likely to bleed in these instances and even women who are only affected by a mild bleeding disorder will suffer more during gynaecological events because their bleeding disorder will contribute to the bleeding.

On the role of the obstetrician-gynaecologist (OB-GYN)

Rezan’s work is very diverse. She is trained as an OB-GYN but also works in the area of women and bleeding disorders and in this capacity she cares for all aspects of gynaecological and obstetric care for her patients. So for instance, when women come to her clinic, she will be able to take care of the prenatal diagnosis thanks to her specialisation in foetal medicine, she will also do gynaecological surgery, and thanks to her obstetrics specialisation, she will be able to look after her patients during delivery. Additionally, she works as a community gynaecologist and as such she is able to provide advice to women on issues such as contraception. Through all of these services, Rezan is able to provide a comprehensive care service to women, which is not always the case in other centres.

On working with other healthcare professionals

Rezan works closely with the nurses in her centre whom she sees as crucial in running the OB-GYN service. In particular, she works closely with a specialised haemophilia nurse who is key in running the women’s service. Nurses are very important in establishing the first contact with the patient and assessing the patients’ needs. Often the nurse will be able to sort out most of the problems by her himself, however whenever the problem is too complex, the nurse will bring it to Rezan’s attention. The nurse is also key in the organisational aspects of the clinic and will liaise with other specialists such as the scanning technician, for example, to arrange prenatal diagnosis.
The family therapist is also a very important healthcare professional Rezan works with as she often has to deal with a lot of sensitive issues affecting, for example, young girls and families. In particular, the family therapist is very helpful in helping to deliver delicate news like being a carrier or having a male baby.

Additionally, Rezan works closely with physiotherapists because many of the women she looks after need help through delivery and later on with other problems. She also works with haematologists and jointly runs the women’s clinic with them. Finally, she works very closely with the midwives as they provide support to her patients during pregnancy, labour and delivery.

On OB-GYN services for carriers and women with bleeding disorders

“Women are women!” states Rezan, meaning that all women, irrespectively of their general health status, will experience some gynaecological issue or another in their lifetime. For women affected by a bleeding disorder, perhaps the menstrual bleeding is the main problem because it takes place every month and is accompanied by other issues such as pain, an irregular menstrual cycle or endometritis (i.e. an inflammation of the inner lining of the uterus). From a scientific standpoint, there is still a lot of work to do to prove the link between being a woman affected by a bleeding disorder and increased bleeding in the OB-GYN area. Nonetheless common sense tells us that it is highly likely carriers and women with bleeding disorders are more affected and suffer more than other women when it comes to OB-GYN problems.

On childbirth in carriers and women with bleeding disorders

For carriers there are two main aspects that need to be looked at when it comes to pregnancy and childbirth. On the one hand they need to be taken care of during pregnancy and childbirth and the OB-GYN needs to look out for physical symptoms such as bleeding. On the other hand, there is the whole aspect of the prenatal diagnosis of their future children.

With regard to bleeding symptoms, it is important to see these women (both carriers and women with bleeding disorders) early on, ideally before the first bleed and certainly before the first period so that the OB-GYN will know what their factor level is and whether they are going to have a heavy bleed. This is very significant because it can be very shocking for a young girl to have a heavy bleed for the first time in her life. It is also important to monitor and prepare women with bleeding disorders for surgery, whether it is a gynaecological surgery or not, because the bleeding symptoms can be significant.

From the genetic point of view, the most important aspect is preparing women, mainly carriers, for the fact that they could carry a male who could be affected by haemophilia or another bleeding disorder. It is then important for Rezan and her team to explain to these women concretely what this means, what are the options for prenatal diagnosis, for delivery and beyond, if they do have a bleeding disorder what are the options are for her child’s treatment.

On access to specialised OB-GYN services in the UK and in Europe

Access to specialised OB-GYN services is variable across the UK. For instance, in Rezan’s centre the situation is good because there is a dedicated clinic, a specialised service and healthcare professionals
who are available to patients. Unfortunately, this situation is not the same everywhere. Most of the haemophilia centres in the UK have good access although there may be some differences across the centres.

She also notes the important role played by the UK Haemophilia Society both in raising awareness through the ‘Talking Red’ campaign and in giving patients information on treatment centres and referring them to specialists (including Rezan’s clinic) when needed.

As for Europe, in general there are disparities in terms of access to treatment and among OB/GYN specialisation for bleeding disorders. This can even occur within the same country where the types of services offered can vary from centre to centre. These differences are also influenced by the level of activity of the local patient organisation and the interest of the healthcare professionals. If the physicians are not interested in women with bleeding disorders and carriers, then these are more likely to be neglected.

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For Rezan equality of access to treatment is the main problem for carriers and women with bleeding disorders.

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On specialised training for OB-GYN

There is no specialised training for OB-GYN to care for women affected by bleeding disorders. Currently, in the UK there is a subspecialised module for medical disorders in OB-GYN that covers all medical problems and the focus will be on more common issues such as diabetes, although haemophilia is also part of this module.

At the Royal Free Hospital, all trainees who have taken up this module are sent to Rezan’s centre for at least a couple of sessions to make them aware of women with bleeding disorders but then again this is not universal and this training should also be added to the general OB-GYN training. Things, however, are getting better. Whereas in the past these women were not talked about, now this area is part of the curriculum for medical students. For example, nowadays the examination of the UK Royal College of Obstetricians and Gynaecologists, which confers the highest qualification for OB-GYN in the UK, also features questions on women and bleeding disorders; something that is in and of itself very encouraging.

On professional societies and international collaboration

In terms of international collaboration, there is in Europe an international symposium for women’s health issues in thrombosis and haemostasis¹, which takes places every two years and is attended by both OB-GYN and haematologists.

There are also other international societies such as the World Federation of Hemophilia (WFH) and the International Society for Thrombosis and Haemostasis (ISTH), both of which have programmes focused on women’s health issues in the area of bleeding disorders. The WFH is more patient-oriented while the ISTH is more scientific and presents ongoing research about women and bleeding disorders. Rezan is currently chairing the ISTH Women’s Subcommittee²; a fairly new committee looking at research priorities, training programmes, raising awareness and developing international collaboration. This committee also develops an almost yearly symposium during the ISTH congress.

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¹ [http://www.whith.org/](http://www.whith.org/)
On the collaboration with patient organisations

The UK Haemophilia Society has a lot of material on their website and is a point of contact for women needing help and information. Therefore if a woman only has access to her local general practitioner (GP) or hospital, she can call the UK Haemophilia Society and they will give her information or transfer her request to Rezan’s centre who can either refer them to the appropriate treatment centre or even welcome the patient in her centre for a second opinion.

Additionally, Rezan is on the medical advisory board of the UK Haemophilia Society who provides professional opinions on the material produced by the Society such as leaflets and other educational publications. Her role consists of reviewing the accuracy and the language of the material produced. Furthermore, some of the nurses working in her centre are actively involved in training courses and in producing posters for gynaecology services and GP practices on women and bleeding disorders. A few years ago Rezan worked with the UK Haemophilia Society to identify the priorities and problems of young girls affected by bleeding disorders.

“Women are women!” states Rezan, meaning that all women, irrespectively of their general health status, will experience some gynaecological issue or another in their lifetime. [...] From a scientific standpoint, there is still a lot of work to do to prove the link between being a woman affected by a bleeding disorder and increased bleeding in the OB-GYN area. Nonetheless common sense tells us that it is highly likely carriers and women with bleeding disorders are more affected and suffer more than other women when it comes to OB-GYN problems.

On the current and upcoming challenges for OB-GYN and bleeding disorders

For Rezan equality of access to treatment is the main problem for carriers and women with bleeding disorders. Unfortunately, a woman with the same disorder could have perfect care in one place and be neglected somewhere else.

The second problem is the need to increase awareness amongst physicians, gynaecologists and even haematologists. In Rezan’s opinion, some healthcare professionals do not fully appreciate the problems of women with bleeding disorders. For example, a woman suffering from a mild bleeding disorder may not exhibit enough symptoms to warrant their attention. Therefore increasing awareness is important.

Finally, “We need more research!” she states. When it comes to women’s problems there is very little that is done and gaining financial support from pharmaceutical companies is very difficult as most women do not need coagulation factor concentrates. However there are many areas needing research efforts and she hopes that companies will come through and provide support for this.

* Dr Rezan Abdul-Kadir is a Consultant Gynaecologist with a specialisation in foetal health at the Royal Free Hospital in London, UK.

** Laura Savini is the EHC Communications and Public Policy Officer.
Key messages from the EHC Annual Conference in Belgrade

By Laura Savini*, Aislin Ryan** and Declan Noone***

This year’s Annual Conference of the European Haemophilia Consortium (EHC) took place in Belgrade, Serbia, from 2-3 October and was attended by over 300 participants representing patients, physicians and industry. The event featured workshops (see pg 17 and pg 19), scientific sessions and industry symposia. Laura Savini, Aislin Ryan and Declan Noone report on the main points given during the scientific sessions, which this year looked at the organisation of haemophilia care in Serbia, gene therapy, women and bleeding disorders, complications in haemophilia and extended-half life (EHL) products.

On haemophilia care in Serbia

The conference opened with a session on different aspects of haemophilia care in Serbia. The session was chaired by Dr Danijela Mikovic, from the Blood Transfusion Institute of Serbia.

Prof Predrag Miljic, from the Haemophilia Comprehensive Care Centre, Clinical Centre of Serbia, gave the first presentation on the management of inhibitors in Serbia. He outlined the programmes in place to support people with inhibitors in the country. This included annual training camps for adults with inhibitors introduced in 2013 by the Serbian Haemophilia Society in cooperation with healthcare professionals from Serbian haemophilia treatment centres. Dr Miljic emphasised the importance of having a comprehensive approach to haemophilia treatment in general and inhibitor management in particular. Among the benefits of the training camps was not only providing education, but also giving an opportunity for patients and comprehensive care team members to interact and build trust.

Next Dr Mikovic spoke about research into rare bleeding disorders (RBDs) and Serbia’s involvement in this. She described the work of the European Network of Rare Bleeding Disorders (EN-RBD)3 and the PRO-RBDD project4, both aimed at collecting data on rare bleeding disorders from centres in multiple countries in a common way in order to investigate a number of areas related to RBDs including their epidemiology, their laboratory diagnosis and genetic characterisation and clinical outcomes, including risk factors for bleeding and the efficacy and safety of treatment products.

3 http://www.rbdd.eu/
4 http://eu.rbdd.org/
On gene therapy

Prof Flora Peyvandi from the University of Milan, Italy and a member of the EHC Medical Advisory Group (MAG), gave a presentation on the current state-of-the-art in gene therapy. She discussed the different delivery systems that are being assessed and their pros and cons. She also discussed ongoing issues such as the factor levels that, despite gene therapy, are still not high enough to free some patients from additional infusions. Additionally, the expression on the FVIII or FIX is short lasting in some trials, inhibitor development is still an unknown and innate immunity to the delivery system for the FVIII and FIX gene. Prof Peyvandi pointed out that there are potential solutions from supressing the person’s immune system at the time of receiving gene therapy to the possibility of different types of delivery mechanisms. She finished by focusing on the current reality. Gene therapy is an emerging viable way to treat FIX deficiency and encouraging results have been achieved in patients, however, despite many efforts, gene therapy for FVIII is still problematic.

On women and bleeding disorders

During this session Dr Roseline d’Oiron from the Haematology Service of the Hôpital Bicêtre in Paris, France, talked about ‘Genetic and bleeding risk in carriers of haemophilia: diagnosis and care,’ while Prof Rezan Kadir (see pg 9), a consultant gynaecologist at the Royal Free Hospital in London, UK, talked about ‘Pregnancy and menorrhagia.’

Dr d’Oiron spoke about the need for improving tests for carriers and highlighted that to achieve this it is important for families, patient organisations and haemophilia treaters to improve communication on the subject and raise awareness. Dr d’Oiron also talked about the different stages and the care needed for carriers as they age. These are as follows:

- In early childhood the bleeding risk needs to be assessed and this assessment should be followed-up in pre-adolescence;
- For adolescents and young adults, families and physicians need to start preparing to discuss the possibility of being a carrier and tests should begin;
- Young adults, with or without a partner, need counselling and to be able to discuss possible pregnancies and delivery options;
- Ageing carriers need guidance and counselling for premenopausal periods, surgeries and other co-morbidities need to be considered.

Prof Khadir spoke about the need for a multi-disciplinary approach and the need for close collaboration between obstetricians, gynaecologists and the rest of the healthcare professionals on the haemophilia team. There also needs to be more awareness of current evidence for treating menorrhagia and pregnancy protocols as well as the variety of options for treatment that are currently available. Prof Khadir emphasized the need for further research and the potential need for specialist clinics for severe and complex cases.
Prof Angelika Batorova, member of the EHC MAG, chaired the session and commented on the fact that the topic of carriers and women with bleeding disorders is very important because it is still a difficult topic for women to talk about within and outside their family. Additionally, some haemophilia treatment centres are unfortunately still ill-equipped to care for women with bleeding disorders and carriers and therefore raising awareness about the topic has never been so important.

**Complications in haemophilia**

The topic was looked at in two different sessions. On Saturday, Prof Paul Giangrande, Chair of the EHC MAG, moderated a session in which Prof Philippe de Moerloose, President of the European Association for Haemophilia and Allied Disorders (EAHAD) and Head of the Haemostasis Unit at the Geneva University Hospitals, talked about ‘Thrombosis in bleeding disorders,’ which focused on thrombotic problems in haemophilia and von Willebrand disease. For Prof Giangrande the take-away message of this session was that treaters need to start to give prophylaxis to people with haemophilia and low molecular weight heparin prior to orthopaedic surgery when there are risk factors. Prof de Moerloose also noted that some of the rarer bleeding disorders are associated with a risk of thrombosis.

The second presentation of this session was given by Dr Luigi Solimeno, orthopaedic surgeon at the Centro Traumatologico Ortopedico in Milan, Italy, who gave a talk on ‘Orthopaedic surgery.’ In his presentation he emphasised the need to prepare the patient well for orthopaedic surgery to keep the risk of bleeding in the immediate post-operative time low. Dr Solimeno also stressed the importance of being aware of early signs of joint infections following the surgery because if the infection is identified early it is easier to treat. Unfortunately, all too often the diagnosis is made too late because signs of discomfort in the knee are easily dismissed.

On Saturday, Prof Michael Makris, member of the EHC MAG, chaired and presented during the second session focused on complication in haemophilia care. In this session Prof Pier-Mannuccio Mannucci, Scientific Director of the Research Hospital Cà Granda Foundation in Milan, Italy, gave a presentation on ‘Ageing and cardio-vascular health.’ Prof Mannucci focused on cardiovascular diseases and co-morbidities in haemophilia. He noted that these co-morbidities are increasing amongst people with haemophilia and can be classified into two groups. The first group is linked to the consequences of the blood contaminations that occurred in the late 1980s and early 1990s in the haemophilia community, such as liver cancer and liver diseases due to hepatitis. The second group of co-morbidities are related to ageing as the haemophilia population has an almost normal rate of life expectancy, which is a fairly recent phenomenon and due to this fact the body of evidence on how to treat people with haemophilia and age-related comorbidities is not very extensive. However, it is still important to have some guidelines to treat people with haemophilia and age-related co-morbidities to manage these patients. Prof Makris noted that a difficult situation to manage is when a patient with haemophilia also has a heart attack and therefore a set of guidelines would be a starting point. Additionally, it is critical to have a close collaboration between haematologists and other specialists. For example, when managing cardio-vascular diseases, close collaboration between haematologists and cardiologists is essential.
Prof Makris, consultant haematologist from Sheffield University, UK and a member of the EHC MAG, then gave a presentation on the ‘Management of inhibitors.’ In his lecture Prof Makris made the point that inhibitors are overall rare and occur in four groups: previously untreated patients, previously treated patients, patients with mild haemophilia and patients with acquired haemophilia. The management of these patients is to treat their bleeds. For this purpose, Prof Makris stressed the importance of listening to patients’ views in their preference for the product as patients may express some preferences towards one particular product or another. Therefore dialogue between the treater and the patient is essential. Additionally, Prof Makris noted that doing surgery in these patients is very expensive and challenging and should only be done in specialised centres. In terms of removing the inhibitors, there are a number of ways of doing this but big international studies have shown that the low-dose and the high-dose regimens produce equivalent results in terms of clearance but the patients who receive the high dose are at a reduced risk of bleeds during the immune tolerance therapy (ITI). Furthermore, there are nine new agents under development to treat patients with haemophilia so the field is going to look very different in the next five years.

*On extended half-life factors*

The last session was on extended half-life (EHL) coagulation factor concentrates. This was a comprehensive look at the different considerations with the impending release of these products in Europe. Dr Manuel Carcao from the Sick Kids Hospital in Toronto, Canada, spoke about his clinical experience so far with the new products. There are four EHL FVIII coagulation factors and three EHL FIX coagulation factors coming to the market over the next few years. He pointed out that with these new products the extension in the half-life of FVIII has been increased approximately by one and half times, while the extension in the half-life of FIX has been increased between three and five fold. This meant, from a clinical point of view, that most patients on FVIII were able to reduce frequency of infusions from every second day to twice a week, and in patients with FIX deficiency from two to three times a week to once a week. In both situations there was less bleeding and bruising and overall patients on EHL FVIII reported being content, while those on EHL FIX reported being extremely content. Dr Carcao pointed out that it was much too early to comment on inhibitor development with these new products. It was also noted that clinical trial doses were higher in some situations than would be currently used in normal practice and when they come to the market more individualisation of treatment will be needed. Prof Paul Giangrande reaffirmed this and the need for tailoring doses based on pharmacokinetics. Going forward there will be no ‘one size fits all’ regimen. Treatment will be individualised and it will be easier to ensure that trough levels of above one per cent are achieved. He also noted that other outcomes are also equally important and the lifestyles of individuals needs to be considered. Compliance with therapy will also be important to fully prove the benefits of EHL products. Furthermore, as these products are new and are, as such the molecules in them are altered from their original state, there needs to be good monitoring through post-marketing surveillance. Prof Peyvandi talked about the need for a common structure for all registries to collect data and enable cooperation between databases. The final discussion point was on the economics of the new treatments. The session looked at how the true value of these new products could be assessed compared to current products. New concepts in the economics in the cost evaluation of treatment were discussed such as a cost per patient per year, a cost per trough level or a cost based on outcome, which would include other services in the haemophilia life-cycle.

*The webcast of the scientific sessions will be available in early 2016 to all Conference delegates. For more information about this, please contact Laura Savini at laura.savini@ehc.eu.*

*Laura Savini is the EHC Communications and Public Policy Officer.*
EHC Annual Conference Workshops: Spotlight on Inhibitors and Women in the Bleeding Disorders Community

By Laura Savini* and Kristine Jansone**

As with every year, the European Haemophilia Consortium (EHC) organised during its Annual Conference two parallel workshops to explore specific topics relevant to the European bleeding disorders community. The parallel workshops are smaller (with approximately 40 participants) and shorter events (with a duration of one and half hours) than the main scientific sessions. They are typically developed to be more interactive and promote an active exchange between different stakeholders such as representatives from EHC National Member Organisations (NMOs), physicians and industry. This year the EHC organised two workshops, one on inhibitors and one on women in the bleeding disorders community. Kristine Jansone reports on the workshop on inhibitors while Laura Savini reports on the workshop on Women in the European bleeding disorder community.

Addressing the needs of those living with inhibitors – new inspiration, ideas and lots of important work to do!

“People with bleeding disorders and inhibitors have different and more complex problems and needs compared to those who have a bleeding disorder but are not affected by inhibitors. Since this is a small group of people, they are usually side-lined from the local community and sometimes also from their National Member Organisation (NMO). Most of the time, there is no special attention and focus on people with inhibitors. However, during the Inhibitor Workshop organised as part of the Annual Conference of the European Haemophilia Consortium (EHC) in October 2015 in Belgrade (see pg 13), there was a strong will and positive energy among attendees, including myself, to start working on inhibitor-related issues,” says Mirko Jokic from Serbia, one of the speakers at the EHC workshop titled ‘Creating a place for people with inhibitors: Building a community.’

This workshop took place on 2 October 2015 during the EHC Annual Conference 2015 in Belgrade, Serbia, and brought together around 40 participants including people with haemophilia, healthcare professionals and observers from the pharmaceutical industry. The workshop aimed to present the EHC’s new European Inhibitor Network (EIN) programme, by showing data yielded from two needs’ assessment surveys carried out by the EHC as well as by determining further steps for the EIN. The EIN programme is made possible by an educational grant from Baxalta.

Despite the short time allocated to the workshop within the busy schedule of the conference, there was great interest, passion and creativity when discussing the situation and the main challenges faced by people with inhibitors as well as the different ways to create a programme to improve them.

Alongside the presentation of the outcomes of the NMO surveys carried out, the speakers, who were three people with haemophilia who have inhibitors, also provided their personal accounts on the needs of this patient subgroup. One of the speakers was not able to attend the workshop due to a bleeding episode, which showed the fragility and isolation of this group within the bleeding disorders community.
community. However, he was able to provide his input in writing via Skype and his message echoed with those of the two other speakers, which was that: regardless of the country or the region people with inhibitors live in, they face similar, if not the same challenges such as access to treatment, socio-psychological issues and questions regarding integration in society.

Teresa Pereira, a mother of two boys with inhibitors from Portugal, stressed that there is a lot of work to be done following the workshop. Additionally she believes that the workshop has generated some very good ideas for working together at a European level and has offered a platform to start an open conversation between all players involved: patients, doctors, researchers and industry.

The workshop participants as well as the whole EHC membership are looking forward to the EIN initiative. In fact, they believe that this initiative will open new doors and create a community for people who have been more isolated. They also see this new programme as a means towards improving advocacy and capacity building within EHC NMOs.

According to Mirko Jokic, it is essential first of all to develop new guidelines and recommendations on how to treat people with inhibitors. This is key to improving quality of life for people with inhibitors across Europe, and beyond. His hope is for the EIN to focus its programmes on increasing awareness both at a national level with the local patient and medical community as well as with all those involved in the organisation of haemophilia healthcare such as government officials and payers. It is only through this targeted advocacy that the issues faced by people with inhibitors can be properly addressed.

Daniel Andrei, the president of the Romanian NMO, also highlighted the great value of the EIN initiative. He believes that creating a special working group to lead this project will facilitate the implementation of the work.

“As the interest in international assistance by the haemophilia community has led to continuous improvements of quality of life, I believe that work on inhibitors will lead to concrete measures, which will help both physicians and people with inhibitors,” he says. “If the situation has improved in the different countries for people living with haemophilia, it has been due to the efforts and actions taken by international organisations such as EHC and the World Federation of Hemophilia (WFH). In Romania, the situation improved immediately after the 2013 EHC Annual Conference where a Memorandum of Understanding was signed between the EHC, the Romanian Ministry of Health and the Romanian NMO. Likewise, we saw an improvement in access to treatment following the Wildbad Kreuth III initiative recommendations and their endorsement by the Council of Europe. I believe that the EIN formation will bring best practices and recommendations for the treatment and care of people with inhibitors, according to their needs.”

Daniel also highlighted the importance of NMOs active involvement in the EIN work to ensure its success. He gives as an example, the importance of several NMOs including the Romanian NMO in the translation of the needs’ assessment survey conducted by the EHC. This was very important assistance in order to ensure that we reach out to as many people with inhibitors as possible. Being able to respond to the survey in one’s own language increases the motivation and ease to fill it in and shows how the support of NMOs is paramount in the implementation of the programme.

The EHC warmly thanks all the participants of the workshop who contributed to its success and invites all the NMOs to get involved in the future activities of the EIN, supporting people who have inhibitors in their communities!
Kristine Jansone coordinates the work of the EHC European Inhibitor Network. To find out more about this programme or to get involved, please do not hesitate to contact her at Kristine.Jansone@ehc.eu. Kristine can be contacted in English, Latvian, French and Russian.

The EHC is delighted to welcome the following volunteers as members of its Inhibitor Working Group:

- Mirko Jokic (Serbia)
- Hannel Kareranta (Finland)
- Oleksandra Stasyshin (Ukraine)
- Christina Burgess (UK)
- Miguel Crato (Portugal)
- Carl Fredrik Gustafsson (Sweden)
- Elisabeth T. Olesen (Denmark)
- Brian O Mahony (Ireland)
- Paul Giangrande (UK)
- Radoslaw Kaczmarek (Poland)
- Flora Peyvandi (Italy)

The Working Group is coordinated by EHC staff members, Kristine Jansone and Amanda Bok. One of the many tasks of this Working Group is to develop and implement the first EHC Inhibitor Summit, which will take place on December 1-4, 2016.

The working of the EIN is made possible through an educational grant from Baxalta.

Women in the bleeding disorders community

The haemophilia community has been traditionally dominated by male volunteers, this is because the condition typically affects men. However, behind each male there is more than one woman who feels equally concerned and is equally involved in the life of people living with bleeding disorders. They are mothers, usually the carriers of the haemophilia gene, sisters who are also potential carriers, wives, girlfriends and partners who share the life of those living with the disease, and daughters who may also be carriers and one day become the mothers of a person with haemophilia. Additionally, bleeding disorders also affect women, although some disorders affect women more than others, such as von Willebrand disease and platelet disorders, there are women who also suffer from severe haemophilia. It is the role of any haemophilia organisation to be representative and inclusive of all types of disorders and of all the different segments of its patient population. Therefore it is then natural that the EHC should expand its activities to focus on the issues of these women living in the European bleeding disorders community as well as inhibitors, another category of patients that has not been given much attention in the past (see article above). This inclusive approach was particularly clear during the last EHC Annual Conference where, besides a workshop dedicated to these women, there was a scientific session fully dedicated to women and one of our youth debates (see pg 70) explored whether women are sufficiently represented within the patient community.
As a newcomer to the haemophilia world, I was extremely pleased to discover how many women are actively involved in this community and it is good to see that most activities of the EHC manage to achieve some gender balance and representation, for instance our Steering Committee includes two women, all of our committees have women sitting on them and most of our National Member Organisations (NMOs) have women involved in their activities.

The idea for the workshop on women in the bleeding disorder community came from Mrs Yannick Collé (see EHC newsletter August 2015), a member of the EHC French National Member Organisation (NMO), l’Association Française des Hémophiles. Within the French NMO, Yannick is the chair of the women’s committee and has carried out work to raise awareness and provide support to women affected by bleeding disorders all across France. Yannick came to the EHC with a proposal to develop an event focused on women and in the process rallied some other brilliant women to join her to develop the content of the workshop; they include Mrs Helen Døsher (Chairperson of the Norwegian NMO – see pg 28), Ms Evelyn Grimberg, a member of the Dutch NMO (see pg 50) and Mrs Christina Burgess, membership officer of the UK NMO (see pg 41).

The workshop was meant to be a conversation starter to explore what was needed and what could be done at a European level to support women in this community. After short presentations from Yannick and the other moderators about what is done for women in their own countries, the participants of the workshop were split into four groups to explore four different questions: What is done in your country? Do you think there are special needs for women? What are your expectations for this workshop? If there is a lack of action in your country how could we work together to help you?

The participants, primarily female patients, though we were also pleased to see some ‘brave’ male patients amongst us who felt equally concerned by the plight of their female counterparts, were split into four groups and had approximately an hour to discuss these questions amongst themselves and to report each group’s findings to the rest of the audience.

What is done in your country?

Participants reported on the type of activities that are being carried out in their countries to serve their female members. It seemed that organising specific women’s events such as weekends, specific meetings or special sessions during the NMO annual meeting or any other meeting was a fairly common activity. Additionally NMOs develop communication tools through which they can disseminate relevant information such as through Facebook pages, information booklets and special sections on their websites. Some NMOs specifically reported maintaining contacts and good working relationships with genetic counsellors.

Do you think there are special needs for women?

Participants definitely saw special needs for women with bleeding disorders, the primary need being access to tailored and specific information, for instance on gynaecological issues, family planning, dietary requirements as well as needing support in dealing with interpersonal relations such as family acceptance about being a carrier and being able to talk about this to their partners and the external world. Some participants also highlighted the need to raise awareness with healthcare professionals who may not be aware of the special needs of carriers and women with bleeding disorders (see pg 9).
Additionally, there was a call for more advocacy to raise awareness of the problems related to women in the bleeding disorder community. Finally, participants noted that there is a need to be able to share experiences with those who live in similar conditions and to gain emotional support from peers.

**What are your expectations for this workshop?**

The primary objective of the workshop for participants was to be able to network and share experiences with those from other countries and gather intelligence on what is done elsewhere. Some were hoping that the workshop would lead to starting a European cooperation. Others wanted to receive more information on novel treatments and how to manage pain and blood loss.

**If there is a lack of action in your country how could we work together to help you?**

Participants were primarily looking to the EHC as a platform through which to network and exchange information and best practices on how best to serve women within the NMO community. There is clearly a demand to increase communication between the NMOs and many see the EHC as a catalyst to do this work.

For Yannick Collé, the main force behind this event, there is a real need to work on women in the bleeding disorders community. ‘Participants were eager to start different actions to serve women in the bleeding disorders community but it was clear that one of the main needs is also to have some free time to exchange about their own experiences. This was unfortunately one of the points in the feedback that we received, which indicated that there was not enough time to discuss. For my part, it was really interesting to see that women but also men were interested in the subject. They wanted to exchange and create actions in their countries and we now have to help them to bring these ideas to fruition,” said Yannick Collé, following the meeting.

Helene Døsher described her participation in the event as follows: “In Belgrade I had the honour of contributing to the women’s workshop, which was an initiative of the French Haemophilia Society and Yannick Collé. After sharing my experience from the Norwegian Haemophilia Society with the participants of the workshop, I was fortunate to observe the following discussions at the tables, which clearly reflected enormous differences in how women’s issues are treated in different European countries. While some countries have women’s issues as a natural part of their attention and agenda, some countries struggle with the fact that their Societies don’t take these issues seriously at all. I was happy to see that relationships were made, and hope to see this facilitate itself in specific measures and projects, that will benefit carriers, women with bleeding disorders and women in the community as a whole.”

The EHC was delighted with the energetic, passionate and engaged conversations that started in Belgrade and will continue to work within its community to ensure that women’s issues find the best place and vehicle through which to be heard and integrated in our collective work at European level.

* Kristine Janson, EHC Inhibitor Programme Officer

** Laura Savini, is the EHC Communications and Public Policy Officer
EHC Leadership Conference: Facilitators’ perspective – building foundations for a stronger membership!

By Kristine Jansone, EHC Inhibitor Programme Officer

“The single biggest way to impact an organisation is to focus on leadership development. There is almost no limit to the potential of an organisation that recruits good people, raises them up as leaders and continually develops them.” - John Maxwell, author and speaker

After years of planning, the European Haemophilia Consortium (EHC) was elated to launch its ambitious new Leadership Conference this year. Held as a pilot prior to the EHC Annual Conference in Belgrade, Serbia, it brought together more than 70 participants and facilitators – and hopefully many more will benefit from the contents of the conference downstream – and was made possible thanks to educational grants from Baxalta, Pfizer and Sobi.

The Leadership Conference aimed primarily to provide NMOs with a platform to enable them to exchange their experiences, build stronger cross-country networks and receive training at different levels, across NMO roles and generations, in order to encourage leadership, networking, community-building, succession planning, alignment between NMOs, and exchange of both best practices and lessons learned. Until now there have been several possibilities throughout the year for NMOs to come together, exchange and learn, however, none until now have offered enough time, depth, substance or broad enough participation from almost all NMOs and at three different levels of leadership.

The conference consisted of three main thematic blocs, focusing on governance and planning, finances and fundraising, and tenders and procurement. The EHC covered a broad range of issues within these three blocks and used a variety of training methodologies. Amongst others, this also served to help NMOs explore what different types of topics and training might be possible in future conferences, should the pilot be voted to continue as a fixed, yearly conference.

The participants of the conference appreciated the possibility to come together and learn, despite the short time and many topics to address, and for many the Leadership Conference was a great intro and teaser to some central issues in the community.
The organisation and implementation of the Leadership Conference was an exciting task for the facilitators: it offered another possibility to get to know EHC members, their everyday challenges and the particularities of their work within the NMOs. It was extremely rewarding to see the vitality, curiosity, engagement and inspiration of the participants.

At the same time, during the conference it became clear that there are big gaps between the different realities of NMOs, due to varying geopolitical contexts, the way in which NMOs are structured, language and culture. Those gaps mean that, sadly, not all people with haemophilia in Europe enjoy the same treatment possibilities and quality of life. Through gatherings like this it is possible to bridge these gaps by increasing the capacity of all NMOs and by using these differences as learning experiences. Only then will all people in the European rare bleeding disorders community be able to speak with one voice.

Participants provided much positive and constructive feedback and there is an apparent need for many of the themes addressed during the conference to be touched upon also in the local contexts of NMOs. Most of all the participants appreciated the possibility to exchange, or as one of them expressed it: “It’s great to be here! I learned a lot from other NMOs and was able to share experiences about my NMO.”

The EHC is already working on the concept and programme of the 2016 Leadership Conference, looking carefully into the wishes and recommendations of the participants and taking into account the interests and needs of its NMOs. It is essential for NMOs to actively participate in the Leadership Conference and to ensure that a variety of ages and backgrounds are represented, thus empowering and providing all the different subgroups of the EHC community with the possibilities to learn, grow and be engaged.

The next EHC Leadership Conference will be held from 10-12 June 2016 in Brussels, Belgium. All EHC NMOs are welcome to nominate up to three participants representing senior leaders, young volunteers and staff (if applicable). Calls for candidates to the EHC 2016 Leadership Conference will be launched in January.

For additional information on this event, please contact Amanda Bok at amanda.bok@ehc.eu.
EHC Leadership Conference: Participants’ perspectives

Ahead of the its 2015 Annual Conference, the European Haemophilia Consortium (EHC) held its first pilot Leadership Conference, which aimed to bring together its National Member Organisations (NMOs) to share experiences and discuss common issues. Declan Noone from the EHC Irish NMO and Iova Ionut from the EHC Romanian NMO give us their impressions of taking part to this event.

Attending the EHC Pilot Leadership Conference

By Declan Noon, Chair of the EHC Data and Economics Committee and Data and Public Policy Specialist at the Irish Haemophilia Society

The first pilot leadership conference organised by the EHC brought together some 70 delegates from 38 countries. The aim of the conference was to gather current leaders from each of the EHC National Member Organisations (NMOs) as well as their leading youth volunteers and staff members to discuss common issues faced by EHC NMOs and to share ideas on how to tackle problems and move NMOs activities forward.

There can often be a difference of opinions and ideas on what services the haemophilia societies should provide to their members. These different approaches also extend to deciding how NMOs should move into the future. It was therefore the perfect forum for EHC NMOs to discuss some of these problematics and to give youth volunteers a better understanding of the different aspects required to establish and maintain a successful organisation. Youth volunteers were also able to take stock of which strengths and capabilities are required to become leaders within the haemophilia community. Throughout the two days, delegates covered areas such as good governance, strategic planning, succession planning, funding of the organisation, relations with pharmaceutical companies and tenders and procurement.

The session on good governance explored the key elements for successful organisations. To launch discussions, participants had to answer questions such as, what is the purpose and strategy of a haemophilia organisation? What internal and external policies affect its work? How are the various tasks to be divided among the people concerned such as volunteers, staff, board members, committee members and so on? Depending on the answers to these questions, an organisation can start to define where it needs to go. The discussions also looked at the sustainability of the organisation focusing primarily on its human resources such as volunteers and staff, and on the importance of good communication with all members. In a group exercise, participants were asked to define the key items for the success of a haemophilia organisation. The outcome was that participation, equity, inclusiveness and transparency were the most important aspects for an organisation to have.
The second session was about strategic planning and its importance within an organisation. Strategic planning allows for a better understanding of the work performed by all involved in a patient organisation as it helps those involved to clarify priorities and change the way of working from ‘reactive’ to ‘proactive.’ In the exercise component it appeared that 86 per cent of EHC NMOs have a strategic plan in place. Of these, 46 per cent of them are set for the next one to two years and 54 per cent are for the next three to five years. Essentially across all organisations all strategic plans were developed by a combination of NMOs’ board members, staff members and committee members. The topic of succession planning for haemophilia societies was also discussed to ensure volunteers and staff are able to take over when key people leave the organisation or change position. The session stressed that this type of change does not have to be a negative experience but can be very empowering and if done correctly, provide volunteers with opportunities to grow personally and professionally towards future role.

There was a very interesting session on funding, which was co-chaired by Mrs Traci Marshall-Dowling from the Irish Haemophilia Society and Prof Paul Giangrande, Chair of the EHC Medical Advisory Group. Traci presented the work on the funding survey carried out by the EHC in 2014, which looked at the different aspects of funding for NMOs and tackled issues such as receiving funding from a single source, getting access to unrestricted funding, which is an essential budget component in any patient organisation for supporting day-to-day activities. The funding survey also highlighted the absence of written policies for a substantial number of NMOs in particular around receiving pharmaceutical funding. Ms Laura Savini from the EHC staff discussed the various pharmaceutical codes that set rules and procedures to ensure that industry can provide financial and in-kind support to patient organisations in a transparent and responsible manner. Accepting funding from companies and maintaining autonomy in the decision-making of a patient organisation is entirely possible, however, it is preferable to seek unrestricted funding from multiple sources. Relations between the pharmaceutical industry and patient organisations should always be transparent and any interaction should be disclosed by both parties in an open way.

The final session of the Leadership Conference discussed tenders and procurement of coagulation factor concentrates and how patients can be more involved in this process. The session highlighted the importance of clinician and patient involvement in the purchase of factor concentrates, which according to EHC-own research leads to a more cost-effective model for haemophilia care. However, speakers noted that patients’ and clinicians’ involvement must not be tokenistic but should amount to meaningful involvement with the potential to influence the process. Additionally, the system should empower patients and physicians and support them to be meaningfully involved. The session also pointed out that in countries where there is specific legislation for purchasing haemophilia products, removing them from the regular re-imbursement model, the outcome of the purchasing process is more cost effective. The session finished with splitting delegates into groups for an exercise on how to assess products with the appropriate criteria. Interestingly while there were many components to the criteria such as safety, quality and efficacy there was a clear understanding that price was an important consideration for the purchase of factor concentrates but it was never weighted more than safety, efficacy or quality.

The Leadership Conference gave valuable insights to the running of a haemophilia society and to the importance of having strong and interested youth to carry forward the work that has been achieved over the last 50 years. It also showed that there are capable, strong and enthusiastic youth in our
community and that between the leaders of today and those of tomorrow we have to work together to maintain the strength of the haemophilia community in Europe.

Volunteering and friendship: Attending the EHC Pilot Leadership Conference

By Ionut Iova, member of Asociația Română de Hemofilie (ARH) – the EHC Romanian National Member Organisation

“It isn’t important to do many things, what really counts is to do them with love.”

Many people would like to change the world but as they do not have the chance to do something great, they give up easily. They forget that you do not need any special skills to do something special for someone, that you do not need material wealth to spiritually enrich somebody, as people only need great love and involvement. It is with these statements in mind that I attended the pilot Leadership Conference organised by the European Haemophilia Consortium (EHC) ahead of its 2015 Annual Conference held in Belgrade, Serbia.

Besides being affected by severe haemophilia, I also developed inhibitors. This means that traditional haemophilia treatment does not work for me. This is one of the reasons that prompted me to get involved in the patient community and to join others with great ideas and visions to change and improve the life of those suffering because in my opinion there is no greater joy in life than to give and help others. Life is full of paradoxes and I have recently discovered the paradox of volunteering, which is: the more you give, the more you receive! In fact, in my limited experience, you generally receive more than you give. You offer help and get appreciation. You say something nice to someone and receive a smile back. You hold out a hand to somebody and make a friend. What can be more valuable than friendship? What can better unify two people gathering for a mutual purpose? I realised all of this and more by attending the Pilot Leadership Conference. The noblest thing in life is to help and I was glad to do that for a couple of days in the great family that is the European haemophilia community.

During those few days, I learned more about teamwork and how it can be purposely used to reach a common goal like improving the quality of life for people with bleeding disorders. I met a lot of new people from different countries, different cultures, with a different past and despite our differences each one of us leads our own fight, each with our own resources, dealing with different problems and pursuing our own dreams. Everyone shared accounts from their own experiences with the disease and we also talked about the medical methods that are used in many parts of Europe. I had the unique chance to personally develop myself and improve, but also to learn new things and perfect my skills for the EHC’s Romanian National Member Organisation’s activities, which I volunteer for.

Beside those people that were at the conference, I learned what commitment, the power of sacrifice and happiness are, and I understood what suffering is. I was overwhelmed with a strong desire to comfort, I felt the joy of finding myself useful there, I was inspired and motivated by every single person and I better understood that a life lived for others is a life worth living. I learned that my role is to be like a mirror that spreads around the light that it receives. I learned that even small actions count as many times there is no need for great actions to make one’s day but to work together for the people with haemophilia.
EHC 2015 Annual Conference, Belgrade, Serbia

By Vladimir Ilijin, President of Udruženje hemofiličara Srbije, the EHC Serbian National Member Organisation

Dear friends,

It is with great pleasure that I want to inform you that earlier in October, the Serbian Haemophilia Society successfully hosted in Belgrade three major meetings of the European Haemophilia Consortium (EHC) that brought together over 35 EHC National Member Organisations (NMOs).

The first meeting was the Pilot Leadership Conference (see pg 22 and pg 24), which brought together a great number of leaders and youth members from many national haemophilia organisations. This event lasted two days and gave EHC members the opportunity to meet and discuss their current work and future initiatives. The interactive workshops tackled three major topics relevant to each NMO such as governance, financing and tenders/factor procurement. Because the Conference was evaluated as successful at the EHC General Assembly it was decided that the Leadership Conferences will be organised every year.

In parallel with this conference, our first regional meeting - Adriatic meeting - was held. It brought together NMOs from Slovenia, Croatia, Serbia, Bulgaria, Bosna and Herzegovina, Albania, Montenegro and Macedonia. Beside NMO representatives, medical experts who treat patients with haemophilia were also invited. The objective was to review the current situation and to improve the status of patients in the region through exchange of experiences among the participants.

The most important of the three events was the 28th EHC Annual Conference with the participation of 36 EHC NMOs, representatives from the World Federation of Hemophilia (WFH), physicians, speakers, representatives of pharmaceutical companies, organisers and volunteers. In total there were 348 participants, the highest number of attendees for an EHC Annual Conference so far. During these two days, there were some great topics discussed in the plenary scientific sessions, workshops and pharmaceutical symposia. We were delighted that eminent physicians from Serbia, Prof Predrag Miljic and Dr Danijela Micovic, were invited to give key addresses during the Conference on the organisation of haemophilia care in Serbia. I would like to thank them for joining this event and putting Serbia in the spotlight.

Of course, a great venue and excellent organisation played a significant role in the overall success of this year’s Annual Conference. I would like to give my heartfelt thanks to all of those who were involved in the past two years in the organisation of this successful event. In particular, my thanks go to the wonderful ladies of the Professional Conference Organisers, the EHC staff, all board members and youth committee members of the Serbian Haemophilia Society as well as to all the volunteers who helped make participants feel welcome and at home in our beautiful city. Finally, I would like to thank the nurses who manned the treatment room during the event.

It is my sincere hope that all participants felt welcomed and had the opportunity to enjoy the unique charm that Belgrade offers to its visitors.

Finally, I would like to congratulate Mr Brian O’Mahony for being re-elected for another four-year term as the President of the EHC. I also want to congratulate the EHC’s Lithuanian NMO for winning the bid

Vladimir Ilijin is the President of the EHC Serbian NMO, the host of the EHC 2015 Annual Conference
to host the 2017 EHC Annual Conference. Finally I wish to the EHC's Norwegian NMO as much, if not more, success in hosting the next EHC Annual Conference in Stavanger from 7-9 October 2016. I hope that they will find the experience as satisfying and reinvigorating as we did.

Thank you everyone for the participation, productive work and warm company.

Sincerely,

Vladimir Ilijin
Serbian Haemophilia Society

EHC Conference Stavanger 2016

By Helene Døscher, Chairperson of the Foreningen for blødere i Norge (FBIN) – the EHC Norwegian National Member Organisation

After a most wonderful, informative and fun weekend in Belgrade, Serbia, where I attended the 2015 Annual Conference of the European Haemophilia Consortium (EHC), it is now time to look ahead towards next year’s EHC Annual Conference. The Norwegian Haemophilia Society is excited to welcome the European haemophilia community to Norway and Stavanger in 2016!

In early 2013 the Norwegian Haemophilia Society decided to bid to host the 2016 EHC Annual Conference. The bid was accepted by the Annual General Assembly at its 2014 meeting for which we were extremely honoured and excited.

The Norwegian Haemophilia Society will celebrate its 50th anniversary in 2016 and what better way to mark this historic moment and raise awareness for our cause than by hosting the biggest gathering of the European haemophilia patient community. In 2016 we will also organise what we hope will be a most festive and memorable members’ weekend to which we will invite, besides our members, representatives from other European countries and healthcare professionals who have positively impacted our cause. Furthermore, we have commissioned a professional journalist to write a commemorative book retracing our history, which will also include personal stories of haemophilia patients in Norway and medical development that occurred in the past 50 years. We are planning to give this book to our members as a gift to mark our jubilee.

We chose Stavanger as the destination for the conference in Norway for many different reasons. Stavanger has easy access from the airport and great infrastructure. Additionally, hotel availabilities and prices on weekends are very good. Stavanger is the oil and gas capital of Norway and the city is used to hosting large and international events. Stavanger is also the gateway to Fjord-Norway, and is surrounded by beautiful nature, full of contrasts. Within a few kilometres you can visit both long, white beaches and long fjords with steep mountains. We hope that the beautiful location and serene nature can contribute to our goal, which is providing the delegates with inspiration and motivation to continue their important work at home in their own countries.
The work towards making the annual congress in Stavanger the best it can be, has already started. Hotels and venues have been chosen, the logo has been made, the programme has been preliminarily set (see pg 78) and we have started to work on recruiting volunteers. The Norwegian Haemophilia Society hopes to see as many of you as possible in Stavanger on 7-9 October next year, and we will do our best to make it a memorable stay for our guests.

Welcome to Stavanger, Norway in 2016

Together through the jungle of economics and access to treatment products

By Kristine Jansone, EHC Inhibitor Programme Officer

In September, the European Haemophilia Consortium (EHC) organised its third and last Workshop on Economics and Health Technology Assessments (HTA). The workshop was held in St Petersburg, Russian Federation, and targeted non-EU Eastern European countries. For this reason, the workshop was given in Russian. Kristine Jansone, the newest EHC staff member, had the opportunity to attend this event to familiarise herself with the topic but also to meet with NMOs as, besides English, Latvian and French, she also speaks Russian. She reports on her experience of learning more about HTAs and economics being a newcomer in the world of haemophilia.

I started working for the EHC in July 2015, coming from a context that had nothing to do with rare diseases or healthcare as such (see EHC Newsletter August 2014). Since then I have learned a lot. Perhaps the biggest learning point so far is that knowledgeable patients are empowered patients, and empowered patients are essential for ensuring appropriate haemophilia treatment and care, both for children and adults. But I have also learned that haemophilia is not a cheap condition to treat, which makes it very difficult to achieve the above mentioned, especially because often the choice is not directly in the hands of the patients.

For this reason, the EHC organises regular patient training workshops on the economics of access to treatment, which is one of the ways for the representatives of the NMOs to become aware of the possibilities to obtain the best possible treatment, as well as to develop the skills to actively participate in the decision-making processes. These, however, are not very simple and for someone new like me it seemed like stepping into a jungle. But together we made it through!

The workshop aimed to provide an overview regarding the processes, principles and main actors involved in the process of obtaining the treatment products for haemophilia, as well as offering a possibility to develop a deeper understanding of the process through learning by doing. The workshop started with a multiple choice test, where the participants could assess their knowledge regarding basic health economics and HTAs thus establishing an individual basis to build on throughout the workshop. The same test was taken again at the end of the workshop, thus offering a possibility to concretely assess the participants’ learning curve. In between, the programme consisted of lectures by various experts, such as health economists, clinicians, patient representatives and representatives from the pharmaceutical industry, each of them presented the different aspects of assessing the value
and cost of treatment. In addition, the participants worked on practical exercises and case studies, which allowed them to exchange their experiences and points of view regarding the situation in their respective countries.

These kinds of workshops are especially important for the Russian-speaking community, which is still rather isolated despite more than 20 years since the dissolution of the Union of the Soviet Socialist Republics (USSR). The transition to participatory democracy, including the patients’ involvement in determining their own treatment, has been difficult and full of obstacles. For this reason it is beyond question that constantly learning and improving is essential, even if it involves struggling through the jungle! Learning, growing and becoming more professional NMOs will eventually help them close the gap between the different levels of access and possibilities of treatment that still exist in Europe.

The EHC Workshop series on Economics and HTA was made possible through an educational grant from Pfizer. The EHC will start a new series of workshops on Tenders and Procurements in 2016. Information about this event will be posted on the EHC website in early 2016.

EHC New Technologies Workshop – Looking on the horizon for novel treatments in haemophilia

By Laura Savini, EHC Communications and Public Policy Officer

The European Haemophilia Consortium (EHC) organised its second Workshop on New Technologies in Haemophilia Treatment in November, which looked at up-and-coming products for the treatment of haemophilia. Fifty participants from some 20 countries gathered in London for two days to learn more about these new technologies and to discuss potential clinical applications. Laura Savini reports.

In late November, the EHC held its second Workshop on New Technologies in Haemophilia Care to present to its members an update on innovations under development (or already marketed in other parts of the world) for the treatment of haemophilia. The objectives of the workshops are multiple: on the one hand participants get an update on theoretical information such as the status of clinical trials for extended half-life (EHL) products, gene therapy, biosimilars, AT3 therapy. The second aspect of the workshop is to provide participants with the opportunity to interact with healthcare professionals and patients who have had first-hand experience of these products. The third objective of the workshop is
to present participants with information on how these technologies could be evaluated from an economic point of view and how the claimed additional benefits would be weighted compared to current products. Finally, the workshop offers an opportunity to bring patients’ representatives together to have a free and honest discussion and exchange on current experiences. The result is an engaging and intense weekend in which participants have access to a trove of valuable information that they can bring back home and adapt to their own national situation. The workshop is not intended for novices and participants are required to have a minimum understanding of economic, medical and scientific principles relevant to the area of haemophilia to be able to fruitfully follow and contribute to the discussions; this is one of the requirements asked of EHC National Member Organisations (NMOs) when nominating their representatives to attend this workshop. Besides patients’ representatives, each NMO was also asked to invite either a physician, a regulator or a payer from their country that would be involved in the introduction of these new technologies on their national market. This workshop was made possible by educational grants from Baxalta and Sobi.

In theory
Participants started the workshop with a highly-appreciated presentation by Prof Flora Peyvandi from the University of Milan, Italy, and a member of the EHC Medical Advisory Group (MAG). Prof Peyvandi passed in review the status of clinical trials for EHL coagulation factor concentrates as well as novel non-replacement products. In addition, she dedicated the final part of her presentation to the post-registration surveillance of these products. It is no secret that this is a topic close to Prof Peyvandi’s heart and that she is a strong believer that comprehensive monitoring of these products will be needed while they are marketed to ensure patients’ safety. In her opinion, clinical trials do not provide enough data to fully evaluate the long-term effect of modified proteins and a coordinated European effort is needed to collect statistical data in a meaningful way. This was echoed by a recent workshop organised by the European Medicines Agency (EMA) on developing haemophilia registries.

Dr Steve Kitchen from the Sheffield Haemophilia Centre, UK, gave a very comprehensive presentation on laboratory assays for EHL products. In short, an assay is an investigative procedure in laboratory medicine to assess the quality, measure the potency and activity levels of a molecule. There have been many debates and questions about whether traditional laboratory assays that are used to measure current coagulation factor concentrates could be used in EHL products. This is important to know for a series of reasons not least including whether patients will receive the correct dosage of coagulation product that is adapted to their own ability to metabolise coagulation factor. Dr Kitchen sits on the Laboratory Science Committee of the World Federation of Hemophilia (WFH) and the topic is widely discussed by the group and colleagues around the world. From preliminary research it appears that traditional (one stage clot assay) does not correctly assess the potency of EHL products and that instead chromogenic assays provide more accurate results on the greatest number of EHL products. Dr Kitchen stressed that it is important for manufacturers to provide data on the results of the different assays used on their products. This also means that there will need to be increased cooperation and dialogues between treaters and lab technicians to ensure that they are aware of what product the patient is on and that EHL products will need different testing. It will be also important to disseminate the appropriate information amongst lab technicians in order to avoid mistakes in testing products and samples. In short, much work remains to be done in this area.
Prof Edward Tuddenham from the University College London, UK, and Dr Savita Rangarajan from the Basingstoke and North Hempshire Hospital in the UK, both gave an overview of developments in gene therapy. Additionally Dr Rangarajan also spoke about biosimilars, which are biopharmaceutical drugs designed to have active properties similar to ones that has previously been licensed. According to Dr Rangarajan, these medicines have, thanks to enhanced production techniques and lower prices, the potential to increase the access to therapies for the global population and to further lower prices of traditional coagulation factor concentrates by creating competition. However, she noted that safety risks will need to be further assessed and in particular with regard to the development of inhibitors.

With regard to gene therapy, we were informed that clinical trials for gene therapy in haemophilia A are still at a very early stage and tests are currently being carried out on animals. For haemophilia B, the testing of gene therapy is more advanced with trials carried out in humans with some success, although the therapy is not always effective. In fact, in some cases the therapy has the effect of transforming severe haemophilia into the moderate or mild form, hence requiring additional infusions. Another issue is that the treatment cannot be administered to those who have developed an immune response to the vector virus, which means that the treatment will not be available to the whole population. Dr Rangarajan also made a presentation on the development of AT3 therapy.

...and in practice

During the first day participants were also able to hear first-hand accounts from the mother of a patient with severe haemophilia who is currently enrolled in a clinical trials for EHL coagulation factors and from a patient enrolled in a trial for gene therapy. The presentations were coupled with lectures from Dr Beatrice Nolan from Our Lady Children Hospital in Dublin, Ireland, and Dr Rangarajan who were following respectively these patients. These sessions were very interactive and moving and participants had the opportunity to ask various questions, within the limits of the clinical trials confidentiality.

Another practical session on ‘Selection criteria and clinical scenarios’ was moderated by Prof Paul Giangrande, Chair of the EHC MAG and Mr David Page, Executive Director of the Canadian Haemophilia Society. The session was engaging and gave participants the opportunity to assess various clinical scenarios and decide which product they would allocate to which scenario. Additionally, Mr Page made a presentation on the experience in Canada with EHL products, which came to the market in 2015. Disappointingly, Mr Page informed the audience that at the moment none of the EHL products that have received marketing authorisation, have reached patients. This is because payers do not consider that the lower frequency of infusion is enough of a significant benefit to pay a premium price. Needless to say the audience was fairly disappointed with the news, considering that what is appraised as a ‘non-significant benefit’ could, for an average severe haemophilia patient, mean anywhere from 50 to 100 injections less in a year and higher trough levels, which would ensure a better protection against bleeds. This is something that is clearly not insignificant to patients with bleeding disorders. However, our participants were not discouraged and this news only increased our participants’ motivations to tackle the next day’s sessions, which looked at the economic appraisal of these new medicines.

On economic appraisal and other considerations

The Sunday morning session started with discussions moderated by Mr Brian O’Mahony, EHC President, Mr Declan Noone, Chair of the EHC Data and Economics Committee and Prof Mike Makris from Sheffield University and a member from the EHC MAG. Mr O’Mahony opened the session with a
presentation on how EHL products can be assessed and mechanisms for their purchase. The discussions also looked at the ongoing question that is debated when considering a future with EHL products, which is: should treaters aim to have their patients infusing less frequently or have them achieving higher trough levels. The audience agreed that ultimately the cost of these new technologies will very much depend on the answers to these questions and what is very clear is that there will be no one-fit-all treatment protocol but there should be a much more personalised approach to each patient’s particular profile – this allows me to use the opportunity to advertise our third Round Table of 2016 to be held on November 28th on patient-reported outcomes in haemophilia. The morning continued with three presentations on different takes on the topic, namely an R&D perspective on the economics of new products, a physicians’ perspective on how these products are assessed and a health economist and payer’s perspective on how these treatments will be assessed.

Dr Glenn Pierce discussed at length the factors that generate costs in the development and production of new therapies focusing on EHL products and gene therapy. Dr Susan Halimeh gave a detailed overview of the current reimbursement systems for haemophilia treatment in Germany and Prof Ken Paterson gave participants much food for thought on how payers approach and determine the price for a new medicine. Unfortunately, as payers have to assess a variety of medicines, they are more likely to use a more standardised approach. This is why patients should be more involved in the process to provide their perspective on what end points are more important and make a significant difference to them. This final presentation highlighted the need for continued advocacy by patient groups to explain the true impact of perceived added value.

“In conclusion
It cannot be overstated how much information participants were provided over the two days and unfortunately it cannot be summarised in a few pages either. The only way for one to learn more in-depth about the topic of new technologies in haemophilia care is to attend the workshop.

To conclude this article, I asked some of the participants to tell me what they took home from this two-day event.

For Declan Noone: “The thing that really stuck with me is the fact that the future looks very bright for people with haemophilia thanks to a variety of new technologies that are being developed. For the EHL products we are on the cusp of a potential shift in the way patients are treated considering new paradigms for trough levels or improved quality of life issues such as frequency of infusion. The key component around these will be efficacy and price. With subcutaneous therapies (bispecific antibodies) the results so far are extremely interesting but going forward there needs to be some clarification on safety and protocols in the event of bleeds or traumas. Gene therapy is very exciting too and brings hope for many people for the future. For me, the key question that needs to be answered is what factor trough level do people with haemophilia want or need to achieve from this sort of therapy. Whilst there are a lot of ‘known unknowns,’ the next five to ten years will be the most exciting era in the treatment of haemophilia in its history.”

Jamie O’Hara (see pg 61), member of the EHC Data and Economics Committee, was struck by the apparent collective determination within the haemophilia community to get optimal treatment.
For Mariette Driessens, also a member of the EHC Data and Economics Committee, all of these developments signal exciting times ahead. “One main message for me was that we, as patients, should be clear and bold about what kind of therapy would constitute significant benefit for us.”

For Radek Kaczmarek, member of the EHC Steering Committee: “What I took away was an observation related to gene therapies, which is that while we tend to think of future treatments as a cure or something close to a cure, the boundary between current and future treatments may be more blurry than we realise. For example, we see patients taking part in haemophilia B gene therapy trials that do not achieve factor IX levels that would allow them a total discontinuation of treatment. Furthermore, few if any currently discussed future treatment options will offer a ‘carefree’ life afterwards. This is particularly striking, especially when compared to the outcome of individuals undergoing liver transplant who are completely cured of haemophilia. Obviously this is not a viable cure option per se but it makes me draw a clear comparison between the two scenarios.’

The EHC will organise a new edition of the event in November 2016. For further information on this event, please contact Laura Savini at laura.savini@ehc.eu.
Mild and moderate haemophilia beats Brussels lockdown

By Laura Savini, EHC Communications and Public Policy Officer

On 30 November, two weeks following the Paris attacks, the EHC held its last Round Table of the year on ‘Mild and Moderate Haemophilia.’ The event, which was meant to take place at the European Parliament, required some last minute logistical re-adjustment and suffered from some late cancellations due to the security situation in Brussels. Nonetheless, participants gathered for some very interesting lectures and fruitful discussions on how to tackle issues impacting patients suffering from mild and moderate haemophilia. A special thanks goes to Mrs Mady Delvaux, MEP (Luxembourg/ S&D) Dr Cristian Bușoi (Romania, EPP) and Prof Paul Giangrande for their support and contributions as well as to all participants who braved the security alert to attend this important event.

Mrs Mady Delvaux, MEP, opened the Round Table

The European Haemophilia Consortium (EHC) held on Monday 30 November 2015, its third and last Round Table of the year on ‘Mild and Moderate Haemophilia.’ The event was supported by Members of the European Parliament (MEPs) Mrs Mady Delvaux-Stehres and Dr Cristian Buși. Prof Paul Giangrande (Chair, EHC Medical Advisory Group) chaired the event and moderated the discussions.

Referred to as the silent majority of the haemophilia community, people affected by mild and moderate haemophilia make up for more than half of the patient population and yet they are vastly under-represented in patient organisations and the condition is often downplayed by patients and non-specialist treaters alike. All of this leads to a lack of health literacy amongst patients as well as limited scientific and clinical research. Although these patients are not severely affected by haemophilia, they nonetheless face some of the same issues as patients with the severe form of the disease including increased bleeding during surgery, the risk of developing inhibitors and potentially developing arthropaties and joint damage. Unlike patients suffering from the severe form of haemophilia, patients with mild and moderate haemophilia do not typically have access to home treatment, which translates into prolonged hospital stays during surgeries and burdensome hospital visits to receive treatment following a bleed.

The Round Table kicked off with a presentation from Prof Paul Giangrande in which he stressed the importance of testing patients with a chromogenic assay to ensure correct diagnosis. The event continued with the compelling personal account of Mr William McCudden, a patient with mild haemophilia who also developed inhibitors. Mr McCudden recounted being diagnosed only in his early 30s and developing an inhibitor a few years ago following treatment received after a ski accident. As a mild haemophilia patient, Mr McCudden does not identify with the patient community as he does not see himself as sick. Nonetheless he feels concerned about not being able to access adequate treatment when travelling because non-specialist physicians are not so familiar with his condition. Mrs Olivia Romero-Lux (EHC Steering Committee Member) gave a presentation on studies carried out in Canada, Denmark and France on the knowledge of people with mild and moderate haemophilia about their condition. The studies reveal that most patients do not see themselves as sick and downplay the disease but in return they also face difficulties in getting access to the correct treatment because of lack of knowledge about their condition, infrequent contacts with treatment centres and not being able to tap into information resources provided by the patient community. The Association Française des Hémophiles (the EHC French National Member Organisation) is currently developing an outreach
and educational programme to increase health literacy and empower people with mild and moderate haemophilia to take better control of their condition.

The second half of the event focused on clinical studies and scientific mechanisms involved in the development of inhibitors for people with mild and moderate haemophilia. Dr Karin Fijnvandraat (University of Amsterdam) and Dr Sébastien Lacroix-Desmazes (a researcher from the French INSIRM) presented on these topics. The main findings of the lectures were that unlike for patients with severe haemophilia, where the risk of developing inhibitors is greater during the first 50 exposure days, people with mild and moderate haemophilia can develop inhibitors throughout their lifetime and the risk is strongly linked, amongst other factors, to the mutation of the FVIII gene. An average five per cent of these patients will develop inhibitors, although depending on the gene mutation the risk can vary as much as from two per cent to 60 per cent. Therefore correct testing and a personalised approach to the treatment of these patients are essential. The final presentation was given by Dr Axel Seuser (Centre for Prevention, Rehabilitation and Orthopaedics in Bonn) on the need to identify silent bleeds, i.e. non visible bleeds, early on and to give patients proper advice in terms of safe practice of sports. Dr Seuser stressed that although there is no hard clinical evidence, he and colleagues witnessed first-hand that the safe practice of sports in people with haemophilia can lead to a reduction of bleeds and preserve joints health. It is therefore essential to keep the haemophilia population active with low-impact activities.

All documents related to the Round Table are available on the EHC website. A detailed report of the discussions will be made available in January. The next Round Table will take place in Brussels, Belgium on 16 February and will be on the topic of inhibitors. For registrations, please contact laura.savini@ehc.eu.

EHC Youth Committee welcomes new members from France, Serbia and Slovakia

By Laura Savini, EHC Communications and Public Policy Officer

In 2014, the European Haemophilia Consortium (EHC) held its first Youth Leadership Workshop aimed to bring together young volunteers active in its National Member Organisations (NMOs) to give them training to better serve their NMOs (see EHC August 2014 Newsletter). Additionally, the workshop aimed to give these young volunteers a sense of the work carried out at European level and ensure that they would meet with their counterparts in other European countries to build a network amongst youth in Europe. The event was successful and led to a second edition of the workshop, which took place last July in Rome (see EHC August 2015 Newsletter).

The idea for European youth-focused work within the EHC was first brought to the EHC by a group of young volunteers that included Michael van der Linde from the Netherlands and Federico Ruiz-Garcia from Spain. The EHC further developed the original concept and secured funds to develop the workshops. A team composed of volunteers, EHC members and staff moderated the workshops, while the programme was developed by the EHC Youth Committee, composed of EHC Steering Committee members, Ms Olivia Romero-Lux and Ms Traci Marshall-Dowling, youth volunteers Michael (the Chair of the Committee) and Federico and EHC staff.
Thanks to the success of the first two workshops, the EHC decided to formalise its youth-related work and to assign a more formal role to this committee. Furthermore, Federico, a member of this committee had to leave to devote more time to his up-and-coming family. These elements combined prompted the EHC to launch a call for candidates to join its Youth Committee, namely for two youth volunteers and one advisor. The Committee received many applications from very eager, energetic and qualified candidates from all over Europe and making the final choice was no easy task. In the end the Committee selected Ms Alexandra Ilijn from Serbia, Mr Ivano Sebest from Slovakia and Mr Thomas Sannié from France to join the committee for a three year term (2016-2018).

Alexandra is a familiar face in the EHC community having attended the first EHC Youth Leadership Workshop in 2014 and several of the EHC Annual Conferences. In her country, Alexandra has been an active volunteer for many years and has contributed to the setting up of a youth committee as well as developing the proposal to hold the EHC 2015 Annual Conference in Belgrade. The Youth Committee saw the relevance of Alexandra’s experience for the work of the EHC. In particular, the Committee believes that Alexandra will be able to share her experience in attracting and retaining young volunteers, one of the main difficulties faced by EHC NMOs.

Ivan attended the last EHC Youth Leadership Conference and was one of the debaters at the youth debate organised during the last EHC Annual Conference. He has a background in health administration and economics and has been an active volunteer in his country for many years. We believe Ivan will make a very positive contribution to the work of the EHC Youth Committee, with his health economics background and thematic interests.

Thomas has been the President of the EHC French National Member Organisation for the past ten years and has solid experience on how to engage with youth in his own country. Furthermore, Thomas has also gained much international experience during his time on the Executive Committee of the World Federation of Hemophilia. Finally, Thomas was involved in the development of the past two Youth Leadership Workshops and was a speaker at these events.

The EHC is delighted to formally welcome these volunteers to its Youth Committee and looks forward to closely working together. The EHC would also like to thank all those who applied to join the Committee. We truly feel honoured to have received so many applications from excellent candidates and hope we will stay in touch on future work and initiatives.

The next EHC Youth Leadership Workshop will be held on 8-10 April 2016. Call for applicants will be launched in January 2016.
NMO News

NMO Profiles: Iceland and Israel

In this issue, to continue our series on Focus on NMOs, we speak to Iceland and Israel.

NMO Profile: Blæðarafélag Íslands – the EHC Icelandic National Member Organisation

Helgi Þór Þorsteinsson* interviewed by Laura Savini**

Helgi Þór Þorsteinsson, President of the Icelandic EHC National Member Organisation (NMO) talks to Laura Savini about the origins of the association, its current status and future projects.

The Icelandic National Member Organisation (NMO) of the European Haemophilia Consortium (EHC), being the Icelandic Hemophilia Society or Blæðarafélag Íslands (the “NMO”), was established in 1977 and its establishment was heavily promoted by Dr Sigmundur Magnússon, the main haemophilia treater in Iceland at the time, who remained involved in the activities of the NMO until fairly recently when he retired. At the time, haemophilia care in Iceland was quite limited so the NMO was established to defend the interests of people affected by the condition, to provide and disseminate information and to liaise between haemophilia patients and the hospital. Besides Dr Magnússon, the NMO was composed of parents of young children and younger haemophilia patients. The NMO became a member of the World Federation of Hemophilia (WFH) in 1978 and a member of the EHC in the early 1990s.

Iceland is a fairly small country with a population of approximately 320,000 people and therefore the haemophilia patient population is quite small. Currently, the NMO has approximately 70 members representing both patients and their families. Although the NMO’s mission is to cater to all bleeding disorders, most of its members are patients, or families of patients, with severe haemophilia A, this being the most prevalent bleeding disorder in Iceland. According to the national registry maintained by the haemophilia centre, there are 63 individuals with haemophilia A (13 severe, 7 moderate and 43 mild) in Iceland, along with 89 carriers of haemophilia A. Iceland is further home to 2 carriers of haemophilia B and 2 carriers of haemophilia C (caused by factor XI deficiency) as well as 35 individuals with von Willebrand disease (out of those 9 are classified as moderate patients while the rest are mild).

There is one main treatment centre in the national hospital in the capital Reykjavik and all patients receive prophylactic and home treatment. The centre runs the national registry and patients are cared for by the chief haematologist. As the level of care has been very good there has been little need to carry out continued advocacy programs for access to treatment in recent years.

At present, one of the objectives of the NMO is to establish an outreach programme to patients who are not members of the NMO. Because of the small size of the population, haemophilia tends to run in a small number of families. The objective of the programme will be to facilitate connections with families that are not yet involved in the NMO, e.g. families that have been affected by a spontaneous mutation. Due to patient privacy issues the NMO is not notified of new patients by the haemophilia and thus is unable to reach out to them directly. The Icelandic haemophilia treatment centre does however inform any new patients of the existence of the NMO and urges them to make contact. The NMO is also liaising with other European NMOs to get some inspiration on how to run their outreach programme.

Seeing that the physical needs of people with haemophilia are, for the most part, well taken care for in Iceland, the NMO has the chance focus on providing support for the emotional needs of its members. In this regard, the NMO wants to focus on the emotional needs of patients on topics such as familiarising yourself with the disease to support overcoming feelings of guilt for carriers.
In the past the NMO advocated for a better standard of treatment in Iceland. The success of such past advocacy, not having been possible without the diligent support of the haemophilia physicians in Iceland, has resulted in the standard of Icelandic treatment being quite high and extensive advocacy not having been necessary in recent years. A recent issue of the level of reimbursement for coagulation factor concentrates, however, demonstrates the necessity for NMOs being ever vigilant when it comes to patient care. The recent situation involved the Icelandic government contemplating increases to the level of patient co-payment for a variety of medicines that were used at home, including factor coagulation concentrates. The Society, along with haemophilia physicians were able to secure a high level meeting with government officials and explained that coagulation factor concentrates were in fact medicines for hospital use and that the reason why they were used for home treatment was to improve the patients’ quality of life and to reduce costs on the healthcare system as home treatment means less hospital visits. The proposed increase in patient co-payments was subsequently withdrawn and the NMO likes to think that its arguments were a point in favour of that decision. With new developments in haemophilia treatments, including extended half-life coagulation factor concentrates, advocacy for patients enjoying the best treatment available may however become an issue in Iceland.

Regarding the organisation of the NMO, it is solely run by volunteers. The board is composed of six members, including patients, parents and relatives and meets on a regular basis to run the activities of the NMO. The NMO organises occasional information meetings for its members in which it invites physicians to give medical lectures on issues related to comprehensive care. With regard to involvement in international activities, the younger members of the NMO take part in the summer camp organised by the Danish NMO as well as the Scandinavian Youth Meeting. Members of the NMO regularly attend WFH meetings, EHC Meetings and the annual Nordic meeting, which is attended by all Scandinavian NMOs. The close cooperation with other Scandinavian NMOs through the annual meeting is important to the NMO as it is an opportunity to exchange and learn from other NMOs who are facing similar issues. The next Nordic meeting will be held in Iceland and therefore this is one project that will take a lot of attention from the Icelandic NMO.

Additionally another interesting development is that one of the pharmaceutical companies developing new therapies for the hepatitis C virus (HCV) will be using Iceland as a testing ground to completely eradicate the virus.

* Helgi Þór Þorsteinsson is the President of Blæðarafélag Íslands, the EHC Icelandic NMO.
** Laura Savini is the EHC Communications and Public Policy Officer.
The Israeli National Member Organisation (NMO) recently underwent deep changes and renewed its membership in the European Haemophilia Consortium (EHC). In this issue we hear from Mrs Talya Shahar, President of ALEH about the Society’s origins, its current priorities and future plans.

The Israeli NMO was first established in 1984 to deal with issues related to patients’ rights and to support its patient community to live a full and independent life. This initiative was led primarily by the Head of Israel’s National Haemophilia Centre and several patients’ families. The main issues at the time of the NMO’s creation were the strengthening of the National Haemophilia Centre and dealing with the aftermath of the tragic blood contamination, which strongly impacted the patient community.

At the moment, the Israeli haemophilia community accounts for some 600 patients who are treated at the national and sole Haemophilia Centre in Israel located at the Chaim Sheba Medical Centre in Tel Hashomer, headed by Prof Kenet Gili. The Haemophilia Centre offers unique and comprehensive services for patients with haemophilia and allied severe bleeding disorders. It also provides within its thrombosis and haemostasis unit, services for diagnosis, evaluation and follow-up of patients with thrombosis, genetic and acquired thrombophilia. The centre also features an anticoagulant clinic and serves as tertiary comprehensive care centre, which strive to have its patients at the core of its services and aims to lead a professional and excellent medical, paramedical and psychological team promoting novel therapies, research and international excellence and leadership in all areas of haemostasis.

Furthermore, the haemophilia centre is a leading internationally known Haemophilia Training Centre (HTC) that strive towards improving patients’ therapies and quality of life. The centre interacts with international colleagues, takes part in clinical trials and is well represented at international congresses.

The Haemophilia Centre also keeps a patient registry and people with haemophilia in Israel generally have good access to treatment, including home treatment, prophylaxis for most children and for half of the adult population as well as inhibitor treatment.

ALEH’s primary objective was and still is to support patients and their families and to achieve this, they offer a wide variety of services to improve patients’ quality of life and to help them and their families cope with the disease.

ALEH works in close collaboration with the Israeli National Centre for Haemophilia and provides comprehensive care to its members. The NMO organises support groups, workshops and scientific meetings to provide further information about different aspects of the disease and how to manage the condition on a daily basis. These meetings are often conducted with the support of local medical specialists such as haematologists, social workers and psychologists.

Currently the NMO has planned to hold a ‘Families weekend’ in which families with children up to eight years of age can meet. The weekend is designed to give parents training on how to infuse their children with clotting factor. Moreover, there will be group discussions with a social worker and a psychologist. This event will also give families the opportunity to meet socially and learn from one another on how to raise a child affected by haemophilia.
The NMO is also organising a Children’s Summer Camp ‘Support and Respite’ to help children and their families to manage haemophilia with confidence and independence. The four-day event welcomes children aged nine to 17 and teaches children how to self-infuse independently. Parents are also welcome to take part in similar workshops on disease management and treatment. The event also features support groups where parents can learn how to ease anxiety and build self-confidence.

The NMO also recently started its ‘Big Brother’ project where newly-diagnosed families are paired up with more experienced ones. This programme encourages strong bonds to be created between families and empower both adults and children.

The NMO is at a renewal phase and as such is currently preparing its strategic plan for the next few years.

Meet Barry Flynn: New Chair of the UK Haemophilia Society

By Barry Flynn, Chair of the Haemophilia Society UK

In late 2015, Barry Flynn began his term as Chair of The Haemophilia Society, the EHC UK National Member Organisation (NMO) bringing a wealth of professional and personal experience to bear on the role at an exciting time for the Society.

I was born in Gateshead, Tyne and Wear, in May 1958, the oldest of three boys. Home was an average semi-detached house in a working-class village, where we attended the village schools and then the local comprehensive. The younger of my two brothers and I both had severe haemophilia, but family life was centred on keeping our childhood as ‘normal’ as possible.

Growing up was generally a happy time, punctuated - but not totally dominated - by periods in hospital or stuck at home. Treatment was blood transfusions, bandages or plaster casts and pain killers, with occasional periods in wheelchairs and callipers and, when things got more serious, the odd emergency ambulance.

Whenever possible, we were kept fit and strong and participated in sport, thanks largely to my dad, a factory worker and the son of a miner, who cycled and ran until he was well into his seventies. Hiking, cycling and youth hostelling, tennis, cricket (with tennis balls!) in the park, cross-country and weight-training were all regular elements of our free time. We also managed to stay abreast of school work thanks to the relentless efforts of my mother, and our whole family encouraged us not to be limited or defined by haemophilia.

As treatment evolved and moved from hospital to home, prolonged periods of pain and incapacitation became gradually consigned to memory, making a host of escapades possible for the first time: falling off motor bikes, playing 'illicit' football games and singing in a band.

A reasonable set of A-level results opened up the adventure of leaving home to go to Liverpool University in 1976, where I re-invented myself, lived life to the full (getting into many scrapes!), secured a first-class degree in biochemistry, and met my wife Julie. We married in 1980 and together we have raised three young adults of whom we are inordinately proud.

After university, I began a career as a chartered accountant with Ernst & Young, initially in Liverpool but later on in Birmingham, Nottingham, London and Manchester, before returning to Liverpool as
Managing Partner in 2002. While having haemophilia has affected some career decisions, such as staying in the UK, and turning down one or two opportunities because of the personal ramifications, I have had a hugely rewarding career with a generally very supportive firm. I became an equity partner in 1994 and enjoyed a number of managerial and market-facing roles, travelling all over the world on business and working with great people.

Since the 1990s, living and working with haemophilia has always been a consideration rather than a limitation, apart from the devastating period when contaminated blood led to most people with severe bleeding disorders - including my brother and I - acquiring hepatitis C, and a large proportion also acquiring HIV. My brother was co-infected with HIV and died in 1991, followed in 1993 by his wife, who had acquired the infection before anyone realised the tragedy that was unfolding.

As middle age approached, some of the damage my joints took as a youngster manifested as arthritis in various joints, leaving me less mobile than in earlier years. I had a knee replaced in 2004 and I also have a fused ankle. And an operation on my elbow is coming up.

I successfully cleared the hepatitis C virus following a year of arduous treatment in 2013-2014. But it was another, unexpected medical challenge that finally led to me taking semi-retirement in early 2015.

I had open-heart surgery in 2004 to replace a congenitally faulty valve, but needed urgent and more extensive heart surgery in 2014. When the surgeon recommended a change of gears I could tell that he meant it, so after 36 years with Ernst & Young, I called time on my career. Except not quite! Having coached and mentored a large number of Ernst & Young partners and directors since qualifying as a coach in 2009, the firm has asked me to continue as an independent coach.

Along with other non-executive and part-time roles, I have also rediscovered interests and hobbies – like drawing, painting and visiting historical sites that had previously been eclipsed by work and family commitments. I had been looking for a way to help improve things for people with a bleeding disorder and those who live and work with them, so when I read an email inviting applications for Chair of The Haemophilia Society, the opportunity seemed perfect.

I can’t think of a better way of using my energy and experience such as working with boards to build common goals and plans, managing change, and advocating, persuading and lobbying on behalf of others. I’m really looking forward to getting started!

The EHC gives Barry a warm welcome in the European bleeding disorders community and wishes him much success in his new role.

**Polish Ministry of Health updates the hepatitis C Drug Programme**

*By Radoslaw Kaczmarek, EHC Steering Committee Member*

Polish hepatitis C (HCV) patients, including several hundred people with haemophilia, have at least seven reasons to smile. That is how many new drugs, including sofosbuvir, the Polish Ministry of Health has stunningly introduced into the Polish HCV Treatment Programme during its latest update, which entered into force on November 1st.
Before this, the last meaningful yet modest change to the Polish HCV Treatment Programme came in last May when simeprevir joined telaprevir, boceprevir and the perennial dual therapy with interferon and ribavirin on the list of available treatments. Now the list also includes dasabuvir, ombitasvir, paritaprevir, ritonavir, daclatasvir, sofosbuvir and ledipasvir. This breakthrough is all the more exhilarating because events of the past months did not exactly promise anything close to happen. Quite the contrary, the country lagged behind others in Europe in providing access to many of the new drugs and the wholesale cost of a 12-week treatment with sofosbuvir was an enormous €52,000. Furthermore, the Polish Health Technology Assessment body issued negative recommendations against a few drugs that have now made the list. Altogether, those developments made patients anything but optimistic.

Today, optimism is what most feel, especially those who failed previous less effective therapies and already heard about success from their peers from abroad who achieved sustained virologic response with the new drugs despite previous failures and advanced damage to the liver. If nothing else, what has just happened in Poland confirms that the HCV drug market changes dynamically. Hopefully, more examples will soon follow.

**Recent advances in haemophilia care in Romania**

*By Prof Margit Serban* and *Daniel Andrei**

The history of haemophilia care in Romania is fairly recent. It started after 1990 with the funding of the Romanian Patients Association (RHA) and its affiliation to the World Federation of Hemophilia (WFH) in 1994 and then to the European Haemophilia Consortium (EHC). This was immediately followed by the inauguration of two of the most important twinning programmes: one programme between the RHA and the Austrian Haemophilia Society and one between the Haemophilia Centre in Timisoara, Romania, and the Haemophilia Centre in Munich, Germany. Both programmes proved to be long-lasting beneficial relationships, giving us models for actions, data for comparing the status and performances of haemophilia care in both countries and supporting RHA in its endeavour for initiating reimbursement of replacement therapy for people with haemophilia (PWH). In 1997 we succeeded in introducing haemophilia on the list of the Romanian National Health Programmes. This was the starting moment for the purchase of factor concentrates, at first with point five International Units (IU) per capita per year, which slowly increased to point eight IU per capita per year in 2007. Unfortunately
after this year the quantity dropped back to point five IU per capita per year as funds were diverted to other disease areas. Thus begun a new twinning programme with the Haemophilia Association of Hungary, which led us to build capacity for development and actions. Thanks to our entry into the European Union (EU), we were finally able to use European standards of care to leverage for access more adequate treatment in Romania. This was accentuated when the Ministers of the Council of Europe endorsed Resolution CM/Res(2015)3, which states that each Member State should be using at least three IU per capita to ensure adequate levels of treatment, pushing Romania to achieve this new goal.

Another turning point in our battle for the improvement of haemophilia care in Romania was in 2013 when we hosted, for the second time, the EHC Annual Conference. As President of the RHA together with the support of Mr Brian O’Mahony, President of the EHC, we changed the attitude of our decision-makers by highlighting the message that replacement of the missing factor is not only life-saving but can also prevent disability. This was acknowledged by both the then-head of our National Insurance House, Dr Cristian Busoi (now a Member of the European Parliament, see page 35) and by our then-Minister of Health Mr Eugen Nicolaescu who declared during the Conference that haemophilia was a priority for the Romanian Health System. What followed the meeting was the set-up of a National Haemophilia Council to define strategies to tackle haemophilia care and the work of this body is still ongoing with positive results: the budget for haemophilia care was substantially increased leading to the introduction of prophylactic treatment for children. Additionally, we are very proud to report that home treatment has finally been approved and implemented by law as it is seen as an indispensable and cost-effective measure. This was achieved thanks to the work of the Health Committee within the Romanian Parliament, the work of the Ministry of Health led by Dr Nicolae Banicioiu and the work of the National Health Insurance led by Vasile Ciurchea. We are looking forward to the benefits that this change will bring in our daily life!

Finally, we would like to thank the EHC for its work in advocating for the cause of PWH in Romania, which still ranks in the last position in the EU with regard to the consumption of factor concentrates. We are thankful for the support of our international family in helping us to lead successful actions to change haemophilia care in Romania.

* Prof Margit is the founder and honorary president of RHA and a member of the Romanian Academy of Medical Sciences

** Daniel Andrei is a patient with haemophilia A and the President of RHA

The Danish Haemophilia Society launches a national telemedicine project

By the Danish Haemophilia Society

This fall, the Danish Haemophilia Society (DHS) launched a national project about telemedicine. The long-term goal is to integrate telemedicine into the Danish comprehensive haemophilia treatment. Through this we are hoping to ease patients’ daily treatment routine, improve access to treatment data for clinicians, and facilitate the dialogue between patients and clinicians.

The DHS initiated this project and to ensure its progress is kept on track, DHS has established a project committee composed of representatives from haemophilia treatment centres, the Centre for Telemedicine and Tele-healthcare from two administrative units in Denmark. The first phase of the project is made possible thanks to funding from Baxalta and the Danish Health Foundation.
Internationally, different telemedicine solutions for haemophilia have already been developed and naturally the findings of these projects have been integrated in our telemedicine initiative. In order to assess the real needs of patients and clinicians in Denmark, the first phase of the project focused on conducting two analyses.

The first analysis focuses on documenting the challenges with haemophilia treatment from the perspective of both patients (including the experience of their relatives and care-takers) and clinicians. Various patients’ sub-groups, including children and their parents, youth, 30-49 year-olds, and the 50+ year-olds, participated in either workshops or interviews as a part of the data collection for the analysis. By mapping these challenges, it is possible to identify which challenges can actually be addressed by telemedicine and which have to be dealt with in a different manner. The second analysis looked at the economic and qualitative potential for reorganising haemophilia treatment. Both analyses were carried out in close collaboration with external consultants and we expect to have the preliminary results in the beginning of 2016. By then, the project will enter the next phase in which the administrative units will decide on the potential for continuing the project with regard to the development and implementation of IT-solutions. The administrative units’ commitment is essential because it is critical that the IT-solutions can be integrated into other national e-health systems already in place, such as electronic health journals.

We hope that this project can stand as an example of the potential of NMOs establishing alliances with various actors and demonstrating that, through collaboration, patients can help to redefine the framework for haemophilia treatment nationally.

Results of a survey among German haemophilia patients concerning their expectations and needs towards the new upcoming extended half-life (EHL) products

By Werner Kalnins, President of Deutsche Hämophilie Gesellschaft (DHG), the German EHC National Member Organisation (NMO)

New extended half-life (EHL) products for the treatment of haemophilia A and B will be launched in the next years in Europe and are expected to improve patients’ quality of life and to increase the adherence to treatment. But actually we don’t know much about the patients’ perspectives on the use of these new products with extended half-lives.

The German National Member Organisation (NMO) of the European Haemophilia Consortium (EHC), the Deutsche Hämophilie Gesellschaft (DHG), was interested in better understanding if there is a real need for EHL products in the haemophilia community and to what extent people with haemophilia (PWH) would be willing to switch to these new products depending on their half-life extension. Furthermore, DHG wanted to learn about the PWH’s expectations and concerns towards these new products in order to adapt its support for the haemophilia community (e.g. patient brochures, patient information via newsletter or haemophilia leaflets). Therefore we planned a survey among all PWHs registered at the DHG.
To get an idea of the main concerns and important aspects of EHL products we first organised five different focus groups composed of adult haemophilia patients and caregivers of children with haemophilia from across Germany. These focus groups were conducted by Dr Sylvia von Mackensen, a medical psychologist focusing on haemophilia care and an expert in questionnaire development. In total 32 PWHs and caregivers participated. The survey revealed that most respondents had almost no information about the new products, they wished more information about dosing and safety had been provided and expected the new products to have prolonged half-lives, better stability and improved manageability. Respondents also wished to receive transparent information about the production of these new factor concentrates. They were willing to change product if the benefits were to be significantly better than current products and if the prolongation of the half-life is in fact maintained compared to the actual product.

Findings of the focus groups helped to develop the survey, including questions concerning the following aspects: 1) Demographic and clinical data, 2) Knowledge about half-life of actual factor concentrates, 3) Attitude towards factor concentrates (a) satisfaction with current factor concentrate, b) expectation of new EHL products, c) willingness to switch to new EHL products, d) required information, e) information sources, and f) ease of therapy.

In January this year we sent out the questionnaire to 1,499 PWHs and 697 responded (46.5 per cent), of which 518 were patients and 177 parents of children with haemophilia. 83.7 per cent had

![Figure 1: Respondents’ satisfaction with their factor concentrate (parents, adult patients)](image1)

![Figure 2: Respondents’ willingness to switch to new EHL products (adult patients)](image2)

![Figure 3: Respondents’ willing to switch to new EHL products (parents)](image3)
haemophilia A, 77.8 per cent were severely affected, 61.4 per cent received prophylaxis and 57.8 per cent used recombinant products. One third of the participants did not know the correct half-life of their current FVIII concentrates and three quarters did not know the half-life for their current FIX concentrates. This is in line with their statements when asked what expectations they have of the new products such as “It would be great to know what half-life means. I have a rough idea, but I don’t know” or “Until today I did not know unfortunately what half-life is, I will inform myself quickly” or “What is half-life”. In general, most of the PWHs were satisfied with their current factor concentrates (see figure 1).

Those PWHs who were unsatisfied with their factor concentrates (four per cent – see figure 1) complained about the short half-life, difficult manageability and the need to store it in the fridge. They mainly expected less frequent injections, efficacy and safety from a new product. Some PWH had quite a positive attitude towards the new products, which can be reflected by the following statements “I absolutely want to have the new factor concentrates” or “A dream for kids” or “Eventually, I will then switch from on-demand to prophylaxis.”

In total, 59 per cent of PWH would be willing to switch to new products if they have a prolonged half-life, provide more security when travelling and have the same safety as their current factor concentrates.

When differentiating parents and adult patients, parents of children with haemophilia were more sceptical than adult patients (see Figure 2); only 44 per cent of parents would switch to new products (see Figure 3).

Reasons for willing to switch to new EHL products included the longer half-life, more security when travelling and same safety of the new product (see Erreur ! Source du renvoi introuvable.4).

<table>
<thead>
<tr>
<th>Reasons for Willing to Switch</th>
<th>Patients N (%)</th>
<th>Parents N (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Longer half-life of new product</td>
<td>391 (87.7%)</td>
<td>131 (89.7%)</td>
</tr>
<tr>
<td>More security when travelling for a short period</td>
<td>301 (67.5%)</td>
<td>89 (61%)</td>
</tr>
<tr>
<td>Same safety of new product</td>
<td>280 (62.8%)</td>
<td>104 (71.2%)</td>
</tr>
<tr>
<td>New product advantages for surgery</td>
<td>251 (56.3%)</td>
<td>66 (45.2%)</td>
</tr>
<tr>
<td>Longer stability outside the fridge of new product</td>
<td>242 (45.3%)</td>
<td>57 (39%)</td>
</tr>
<tr>
<td>Better efficacy of new product</td>
<td>217 (48.7%)</td>
<td>61 (41.5%)</td>
</tr>
<tr>
<td>New product beneficial for doing sport</td>
<td>204 (45.7%)</td>
<td>86 (58.9%)</td>
</tr>
<tr>
<td>Easier application of new product</td>
<td>147 (33%)</td>
<td>48 (32.9%)</td>
</tr>
<tr>
<td>Lower price of new product</td>
<td>86 (19.3%)</td>
<td>30 (20.5%)</td>
</tr>
<tr>
<td>Motivated to switch to prophylaxis with new product</td>
<td>63 (14.1%)</td>
<td>12 (8.2%)</td>
</tr>
<tr>
<td>Sufficient experience in practical use</td>
<td>62 (13.9%)</td>
<td>39 (26.7%)</td>
</tr>
<tr>
<td>Other reasons</td>
<td>15 (3.4%)</td>
<td>10 (6.9%)</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Reasons for NOT switching to a new product</th>
<th>Patients N (%)</th>
<th>Parents N (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Fear of inhibitor development of new product</td>
<td>62 (66%)</td>
<td>65 (89%)</td>
</tr>
<tr>
<td>Fear of uncertain safety of new product</td>
<td>59 (62.8%)</td>
<td>53 (72.6%)</td>
</tr>
</tbody>
</table>
No side effect of current product &dnbsp; 51 (54.3%)&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&nbsp;&n...
the information they require to be able to make a decision and from whom they wish to receive this information.

- The majority 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 of their current product.

- We already presented these findings to the members of the DHG’s medical advisory board and it was suggested to undertake the following initiatives:
  
  o Develop and easy-to-understand patient brochure with the information required by PWH
  o Prepare a neutral (i.e. not from the pharmaceutical industry) patient presentation for HTCs
  o Publish the results in the DHG’s haemophilia leaflets
  o Publish the results in national and international journals

- These findings are very helpful for us to optimally prepare information materials on the new upcoming EHL products taking into account the needs and requirements of our members concerning the type of information to provide and how to disseminate it.

- We believe that there is a real opportunity to repeat the same initiative in other countries to see whether PWH have the same expectations and needs towards the new upcoming products with extended half-lives. We think that running a similar survey in other countries would help patient associations and physicians to cater to the information needs of their members about the new upcoming products. In our opinion this understanding of different patient needs from different 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 we used so that it can be adapted to your national needs. Please feel free to contact us using the following email address dhg@dhg.de

Latvia Haemophilia Society receives recognition from Ombudsman

By Baiba Ziemele, President of Latvijas Hemofilija Biedrība, the EHC Latvian National Member Organisation

The Ombudsman of the Republic of Latvia organised, together with the National Library of Latvia and the Latvian Organisation for People with Disabilities and their Friends ‘Apeirons,’ a contest to recognise the efforts of various Latvian non-profit organisations who advocate on behalf of people with disabilities.

 Fifty-two organisations entered the contest and seven were nominated and to our greatest surprise the Latvian Haemophilia Society was recognised with a nomination under the category ‘Loudest Voice.’ This category included organisations, which made an active and effective contribution on behalf of people with disabilities to defend their rights and interests before the state and local authorities. Other categories included: ‘Promoters of Employment,’ ‘Promoters of Digital Inclusion,’ ‘Service Providers,’ ‘Education Facilitators,’ ‘Social Campaigners,’ and ‘Advocates for Children with Disabilities.’

The award ceremony took place on December 3rd in the newly built building of the National Library on the coast of Daugava in Riga, the capital city of Latvia. This was the first edition of this contest, which will now be held annually.
The Ombudsman of the Republic of Latvia is an official elected by the Parliament whose main tasks are to promote the protection of human rights and ensure a legal and expedient State authority, which observes the principle of good administration.

More information about this contest can be found here: www.tiesibsargs.lv

Cycling to Stavanger: eight Dutch volunteers will cycle 900 kilometres ahead of the EHC 2016 Annual Conference to raise awareness for bleeding disorders

By Minette van der Ven, Board Member of Nederlandse Vereniging van Hemofilie-Patiënten (NVHP) – the EHC Dutch National Member Organisation (NMO)

Most people do not know about bleeding disorders, let alone realise what being affected by them concretely means in one’s daily life. To me, having a bleeding disorder is synonymous with spontaneous internal bleeds primarily in the joints and muscles but also in the mucosa as well as external and more visible bleeds, for example after surgery or after an accident. For women affected by a bleeding disorder it also means having heavy menstrual bleeds. The lingering cause of all of these bleeds lies in a genetic defect, which results in a lack, low level or malfunctioning of a protein or platelet involved in the blood coagulation process. Unfortunately, without access to proper treatment these bleeds can be life-threatening and debilitating. Furthermore, prophylactic treatment has also a long-term psychological effect and physical impact on those who receive it throughout their lifetime.

Getting accustomed to the treatment for bleeding disorders requires learning a lot of new information, which is fairly complex. This is particularly true if patients want to be able to understand how the treatment works and impacts their body. To lead a normal life, one needs to continuously have access
to proper treatment and medical care throughout because in fact bleeding disorders, besides being rare conditions, are also chronic and life-long. Luckily the Netherlands offers adequate treatment and care for most, but not all, people affected by bleeding disorders making life easier for people impacted by them. Unfortunately though, this has not always been the case. In the past, for example, thousands of patients were infected worldwide with HIV and hepatitis B and C from their treatment. Therefore it is understandable that anxiety is ever present at the back of the minds of people with bleeding disorders and that our members are constantly vigilant about the safety and access to treatment. Furthermore, there are still a lot of undiagnosed people with a bleeding disorder who suffer from symptoms such as easy bruising, heavy menstrual bleeds and wounds that do not heal fast.

In September 2016 eight patients with a bleeding disorder will cycle from Utrecht, the Netherlands to Stavanger, Norway in time to reach the 2016 Annual Conference of the European Haemophilia Consortium (EHC – see page 28). Each patient will travel the full distance, which means that over 10,000 kilometres (km) will be covered in total both by bike and by boat. The full journey is approximately 1,300 km, 900 of which will be covered by bike. A cycling team composed of eight cyclists and two team captains is planning to complete the journey in two and half weeks with an average of some 85 km travelled each day. It is our estimate that collectively we will burn over 200,000 calories!!!

This project is called Bloedverwanten, which literally means blood relatives and its objective is to raise awareness about bleeding disorders. Therefore we have put together a team that is representative of the whole range of bleeding disorders including: haemophilia A and B, von Willebrand disease, Bernard Soulier and Glanzmann Thrombasthenia.

The Bloodrelatives cycling team. From left to right (upper row): Chris van den Brink (team leader), Robbert-Jan ‘t Hoen, Stephan Meijer, Fred Cuypers, Manon Degeeaar-Dujardin, Ad Schouten (team leader and President of NVHP) - From left to right (lower row): Manuel Baarslag, Minette van der Ven and Evelyn Grimberg. Jeshua van Deijck who is also on the team is not featured on the picture. (photo courtesy of NVHP).

In the past few years there was a lot of attention given to chronic diseases (I am sure everyone will remember the ice-bucket challenge to raise awareness for amyotrophic lateral sclerosis (ALS) and I thought to myself that it’s important to tell the wider public: friends, colleagues, family members or even complete strangers, what a bleeding disorder is and how it impacts one’s life. Even if medicines
are available (and that’s a big if), it means that you need to self-infuse every couple of days to administer the medicine. With prophylaxis that can represent something like well over 100 injections a year! If on the other hand there is no treatment available, you are at permanent risk of physical damage and maybe even death. It’s important to reach a wider audience and to talk about this condition and what better way to do this than by showing to the world what can be achieved by people with bleeding disorders receiving proper treatment and being in good shape both physically and mentally.

Therefore, through this journey to Stavanger, we hope to gain more recognition, to raise awareness and increase capacity within the bleeding disorder community. We also want to send out a message to our members that sport or moving is good for our health even if we are affected by a bleeding disorder. This message will be in fact the focus of the Dutch Haemophilia Society next World Haemophilia Day Campaign. Finally, we are hoping to collect some funds for our NMO and for other countries where the treatment of bleeding disorders still remains to be improved.

We are convinced that we can make this project a success, but how great would it be if we can make this project international. We therefore invite other EHC members to join us and to get involved. Maybe you want to ride to Stavanger with us? Maybe you just want to join us for a day? Maybe you want to come and meet us at the ferry and ride the last stretch with us (that is approximately 25 km)? Do you have any other ideas, please do let us know!

Learn more about Bloodrelatives:

- **Consult the website:** [http://bloedverwanten.eu/EN/index.html](http://bloedverwanten.eu/EN/index.html)
- **Check out their Facebook page**
- **Every month the team members will perform a challenge to get ready for the ride the will post on the website and Facebook.**
- **Watch videos on the Youtube Channel** (all videos are subtitled in English)
- **Get in touch by writing to** minette@bloedverwanten.eu or to m.vanderven@nvhp.nl
New York Marathon: Eight Italians with haemophilia cross the finish line and run 42 kilometres in the Big Apple

By Federazione delle Associazioni Emofilici (FedEmo), the Italian EHC National Member Organisation

In early November eight Italian patients with haemophilia took part in the 45th New York Marathon, demonstrating that people with haemophilia can do anything when setting their mind to it and having access to adequate treatment.

Mark these names: Francesco Fiorini, Enrico Mazza and Luca Montagna are three of the eight Italian haemophilia patients who took part in the 45th New York Marathon. Thanks to the project ‘Marathon’ from the FedEmo, the Italian National Member Organisation (NMO) of the European Haemophilia Consortium (EHC), these three haemophilia patients crossed the finish line of one of the most renowned sport events in the world turning their dream into reality. Francesco, Enrico and Luca were not the only haemophilia athletes to take part in the competition. They were, in fact, joined by another five haemophilia patients with knee prosthesis who were monitored by Dr Pier Luigi Solimeno (see pg 13).

“We are extremely satisfied with this outcome and we believe that this achievement concretely shows to all other haemophilia patients that it is possible to bend the rules of sport practice,” explains Francesco Fiorini with much enthusiasm and satisfaction. “We have achieved a much sought-after goal,” echoes Enrico Mazza while a disbelieving Luca Montagna describes the experience as ‘having conquered the marathons of all marathons being held in one of the most breath-taking settings.’ He describes the training as being ‘incredibly hard and taking a real physical toll.’ Nonetheless he is proud to say his finishing of the crossing-line not only brings him personal satisfaction but was also dedicated to all of those who supported him throughout the project. “I am proud to be part of this group of eight very different people that managed to finish the New York Marathon! We may be all coming from different walks of life but we very surely united and motivated by a common objective: to improve the image and approach to our pathology,” states Luca Montagna.
These eight marathoners, all of them affected by haemophilia, took part in the race with the Sa.Me.Da. ® L.I.F.E. ® wristband: a technological support that guarantees more safety in case of medical emergency. This wristband allows medical staff to immediately access the identity of its wearer and his/her medical records directly on the scene of any accident with the support of a smartphone or computer. The wristband also highlights all essential information to be taken into account during surgery and emergency treatment.

By crossing the finishing line these athletes have accomplished something that until a few years ago would have been unthinkable. They have participated to demonstrate that haemophilia and regular sport practice go hand in hand.

“The participation of our athletes in the New York Marathon demonstrates that in spite of being affected by haemophilia, when there is determination, good physical preparation and adequate treatment, no goal is too high,” states Cristina Cassone, President of FedEmo (see EHC Newsletter April 2015). “This is very much in line with our last World Haemophilia Day theme, which was dedicated to sport. Our hope is that with the Sa.Me.Da. ® L.I.F.E. ® wristband and with adequate guidelines on safe access to sport practice, which are currently being prepared together with the Italian Association of Haemophilia Centres, sports medicine specialists and the Italian National Olympic Committee; people with haemophilia who are in the right physical state will feel more confident and encouraged to practice sports,” continued Cristina.

The initial objective established by FedEmo’s project ‘Marathon,’ launched in 2013, has been reached. The FedEmo marathoners were supported by the sports professionals from the Marathon Centre in Brescia and were guided by one of the greatest trainers of running history, Mr Gabriele Rosa, a cardiologist and sports doctor who has trained countless athletes for major international sports competitions.

The Marathon project has been strongly promoted by FedEmo with the final aim of encouraging sport activities within the haemophilia community. Indeed, the theme of the 2015 Italian WHD was ‘The new challenge of sport activities in PWH’ and the aim was to define national guidelines to guarantee equal access to sport (in some cases also at professional level) regardless of the haemophilia status but taking in consideration the actual physical status, that has been dramatically improved thanks to the availability of replacement therapy.

Therefore, the achievement reached by our athletes has generated strong enthusiasm and optimism within the haemophilia community. Last but not least, this success will hopefully persuade young people with haemophilia that it is possible to live a normal life despite this condition.

*English translation by Laura Savini*

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5 Safety Medical Database (Sa.De.Ma.) Life (Local Infomed For Emergency) is a project developed by FedEmo consisting of wristband with USB key providing immediate information to medical staff about the patient and its medical history. Find out more here (in Italian only).
Feature Articles

Improvement through cooperation: the EHC partners up with European physicians and WFH to improve treatment and care of bleeding disorders in the Baltic region

Earlier in October representatives from the European Haemophilia Consortium (EHC), the World Federation of Hemophilia (WFH) and the European Association for Haemophilia and Allied Disorders (EAHAD) went on a joint visit to Latvia and Estonia to assess current treatment practices and existing infrastructure to care for people with haemophilia and other bleeding disorders and provide support to local patient organisations to advocate for improvement. Baiba Ziemele, President of the Latvian EHC National Member Organisation (NMO) and Killu Kaare, President of the EHC Estonian NMO report.

Latvia receives valuable EAHAD-EHC-WFH expert advice

By Baiba Ziemele, President of Latvijas Hemofilija Biedrība – the EHC Latvian National Member Organisation

In late October Latvia welcomed high-level representatives from EAHAD, EHC and WFH. These experts in the field of haemophilia visited Latvia to witness the state of haemophilia care and to provide advice for potential improvement. Unfortunately, Latvia still falls behind in terms of providing access to treatment for haemophilia and other bleeding disorders with one of the lowest consumptions of International Units (IUs) of coagulation factor concentrates in Europe. Furthermore, despite 20 years of advocacy efforts, the Latvian haemophilia patient organisation still struggles to achieve meaningful collaboration with leading Latvian doctors in the field, which endangers the overall situation of patients with bleeding disorders.

The delegation in Latvia. From left to right, Prof Philippe de Moerloose (President of EAHAD), Mr Alain Weill (WFH President), Ms Baiba Ziemele (President of the Latvian NMO) and Mr Brian O’Mahony (EHC President). One of the goals of the visit was for these experts to speak with representatives from the Ministry of Health to convince them to involve patients and haematologists in the selection and reimbursement process for treatment products. Unfortunately, the Ministry was not able to appoint anyone to meet with this delegation despite month-long efforts to organise the meeting and therefore the meeting was cancelled. For our members, this indicated a lack of commitment by the Ministry of Health towards its patients as, in fact, these types of situations do not only occur with our organisation but also
frequently with other patient organisations as well. Furthermore this situation highlights a lack of willingness from our government to listen to international experts about best practices in the management of haemophilia. In early December 2015, the government resigned again, giving weak hopes that there will be a new Minister of Health by 2016 who would be more patient-friendly, able to speak in English as to communicate with high-level delegations, and willing to hear our advocacy requests for the creation of a National Haemophilia Council and for the involvement of patients and physicians in the purchasing process of factor concentrates.

Another goal of the EAHAD-EHC-WFH visit was to provide training for healthcare professionals and patients on different aspects of comprehensive care of haemophilia, and this was extremely successful. We organised a half-day workshop that took place in the Children's Clinical University Hospital in Riga and had great attendance. All treating haematologists from the adult and children’s haemophilia treatment centres (HTCs) participated as well as nurses, laboratory specialists, physiotherapists and trainees. We also had officials representing the Ministry of Health and National Health Service attending this meeting and they all acknowledged the value and relevance of various presentations given by Mr Brian O’Mahony (EHC President), Prof Cedric Hermans (EAHAD Vice-President), Prof Riitta Lassila (EAHAD Executive Committee Member) and Dr Sébastien Lobet (EAHAD Physiotherapy Committee Member). All the workshop presentations were made available to participants in both Latvian and English for further reference. This workshop allowed us to create new relationships and ensure that healthcare professionals have independent, state-of-the-art knowledge on the global developments in the field.

The next steps after this visit include undertaking further work to establish a National Haemophilia Council. The Latvian Haemophilia Society is also working on new tools to monitor the treatment market and provide all stakeholders with scientifically-relevant data on treatment use and effectiveness. We believe this will help us ensure more tailored treatment for all people with bleeding disorders in Latvia, including 150 people with haemophilia, over 100 people with von Willebrand disease and six people with Factor VII deficiency. We also want to convince key doctors and Ministry of Health officials to pay more attention to our problems.

*The Latvian Haemophilia Society expresses sincere gratitude to the representatives from EAHAD, the EHC and the WFH for bringing sunshine and support, and for encouraging us not to stop improving the lives of people with bleeding disorders in Latvia.*
Estonian patients with bleeding disorders need an inter-hospital comprehensive treatment centre

By Killu Kaare, President of Eesti Hemofiliauhing - the EHC Estonian National Member Organisation

In late October, leaders from the WFH, the EHC and the EAHAD visited Estonia. The aim of the three-day visit was to learn more about the treatment situation of patients with bleeding disorders in Estonia and how this can be supported.

The delegation visited the Tartu University Hospital, the North-Estonia Medical Centre and the Tallinn Children’s Hospital. The delegation found that the hospitals have good facilities to ensure high-quality treatment to patients with bleeding disorders. In order to offer the best management to all patients, however, it was their recommendation to form an inter-hospital treatment and counselling centre (Comprehensive Care Centre), whose first task would be to set up a national registry of patients. In fact, this would ensure equal treatment availability all over Estonia.

In Estonia, there is no single registry of patients with bleeding disorders and diagnosis is lacking, but taking international statistics into account, there are a total of about 400 patients with bleeding disorders in Estonia of which approximately 250 have a diagnosis. As it stands today a patient with haemophilia in Estonia may not receive adequate treatment in places other than the hospitals in the capital city, Tallinn, or in the southern part of the country around Tartu, the second largest city in the country. The creation of a national haemophilia registry would ensure that if the patient's electronic card is opened in any medical institution, the diagnosis will be visible and ideally would also display a telephone number for consultations. Currently the doctor or nurse does not have such a central place to turn to for more specific advice.

Prof Riita Lassila from the Coagulation Disorders Centre in Helsinki and member of the Executive Committee of EAHAD, described the way her centre in Finland functions: "The situation today in Estonia is where Finland was 15 years ago. By gathering the information together in one place, we ensure equal opportunities for the patients, irrespective of their place of residence," she explained. She added that is imperative because the treatment of orphan diseases is complex and a non-specialised treatment regimen may be fatal to patients with haemophilia. The availability of information would be ensured with the establishment of a single telephone line or homepage where additional information could be found by physicians and patients.
On the second day of the visit, executives of the three hospitals and the Health Insurance Fund met with the Minister of Health and Labour Mr Jevgeni Ossinovski to discuss practical steps towards the implementation of the inter-hospital comprehensive care centre and registry. As professional experts Dr Edward Laane and Dr Ines Vaide, the Estonian leaders of the Helsinki-Tallinn WFH Twinning programme, were included in the meeting. All parties acknowledged the need for the establishment of a treatment and counselling centre. Due to the small population of Estonia, the visiting experts recommended that this treatment and counselling centre should combine state-of-the-art treatment for both haemophilia patients and patients with other bleeding and thrombosis disorders in order to best allocate resources. The same opinion was also shared by University of Tartu Hospital Haematology-Oncology Clinic director Prof Hele Everaus.

The high-level visit was organised by the Estonian Haemophilia Society, the haemophilia patient organisation, which is now liaising with the Ministry of Social Affairs, the above-mentioned hospitals, the physicians and the Health Insurance Fund to identify the necessary steps towards establishing this centre. According to the meeting participants, the establishment of the centre is planned for next year. Delegates from the EHC, the WFH and EAHAD committed to following-up on the situation to ensure there is progress.
Researcher spotlight: Dr Gianluigi Pasta

Dr Gianluigi Pasta* interviewed by Laura Savini**

In this edition we start a new column in which we interview a researcher working in the area of comprehensive care in haemophilia and other rare bleeding disorders. For this first edition, we interviewed Dr Gianluigi Pasta an orthopaedic surgeon working at the Orthopaedic and Traumatology Department, Fondazione IRCCS Ca’ Granda, Ospedale Maggiore Policlinico, Milan, Italy. We asked Dr Pasta ten questions to learn more about him and his research focus.

1. What are you currently working on?

My personal ambition is to become an expert in musculoskeletal health for people with congenital bleeding disorders and as such I am led to work on different topics such as clinical and instrumental assessment by, for example, using new methods such as ultrasound HEAD-US score. This is a particular hot topic in the haemophilia world at the moment [Martinoli C et al. Thromb Haemost 2013; 109(6): 1170-9] (see articles on pg 6 and 35) as it is becoming increasingly difficult, in particular since the advent of prophylaxis from early childhood, to detect bleeds and micro bleeds with traditional methods. Nonetheless it remains key to detect and treat bleeds early on to ensure joint health. I have been working closely on this with Prof Carlo Martinoli from the University of Genova from whom I have been learning a great deal.

I also work on surgical and non-surgical options for joint problems. It’s very important to consider non-surgical options before considering surgical options because surgery may not always work. Amongst various non-surgical methods there is, for example, rehabilitation through physiotherapy (see pg 6), the use of orthosis or a change in the lifestyle of the patient, for example by changing the type of physical and or daily activities undertaken by the patient. With regard to surgical methods, I have been focusing in particular on ankle surgeries such as ankle arthroplasty and ankle arthroscopy.

Another one of my research interests is rehabilitation and occupational therapy on which I work primarily with older patients [Pasta G et al. Haemophilia 2015; 21(6): e526-554]. For this particular topic, I am working with the Engineering Department of the University of Milan to develop video games that can be used to improve adherence for patients [Pasta G et al. Haemophilia 2015; 21(6): e525-554].

Finally, I am also focusing on pain management.

2. What does your average day involve?

Seventy per cent of my time is dedicated to clinic work and seeing both in- and out-patients. I work both in the hospital wards and in the operating theatre. The rest of the time is dedicated to research and education activities and my primary interest for these activities is haemophilia. In Italy, there are many hospitals providing haemophilia services but, unfortunately, not all of them can offer all medical services involved in haemophilia care. Therefore, some of my work involves visiting these centres to give consultations on orthopaedic surgery and on how to manage patients with bleeding disorders affected by orthopaedic problems.

3. Why is your work important?

In haemophilia, arthropaty strongly correlates with a reduction in quality of life and increased pain for people suffering from it. Proper orthopaedic management can make a strong impact and positively influence the quality of life of our patients. Furthermore, it can prevent and cure disabilities...
and this is a major achievement.

4. **What do you hope the impact of your work will be?**

My concrete hope is that we, as orthopaedic surgeons and musculoskeletal experts, can be more involved to prevent and detect early signs of arthropathy by means of early clinical and instrumental orthopaedic assessment. In doing so, I hope that we can maintain joint health in the long-term and perhaps avoid surgery altogether.

5. **How did you come to be working in this field and on this topic?**

As if often happens in life it was by chance. I was invited to prepare a lecture on haemophilic arthropathy and then invited to take care of patients with haemophilic arthropathy. So, I started to work in this medical area. Working with people affected by a chronic disease has been very special because it allows to develop a strong relationship with our patients because one can follow them throughout life. In orthopaedics you typically have two scenarios, either you are a good orthopaedic surgeon in which case you treat the problem of your patient and then you do not see him/her again because he or she has healed or you are a bad orthopaedic surgeon in which case the patient will not come to see you again. With haemophilia you follow patients from a young age until adulthood and beyond.

Additionally what is really enriching in this area of work is the multidisciplinary approach to the treatment, which brings me to work with many other specialists such as for example Prof Pier Mannuccio Mannucci from the University of Milan. This collaboration has really taught me a lot in terms of improving my scientific methods, becoming involved in research, being able to publish and generally learning more about different aspects of the treatment of haemophilia.

Additionally I find the atmosphere of the World Federation of Hemophilia (WFH) Musculoskeletal Committee, of which I am a member, very collaborative. It is clear that all those involved in this committee have patients’ best interests at heart. Furthermore being involved in the work of this committee has exposed me to various countries in which there is still much improvement needed in terms of orthopaedic care.

6. **What is the most frequently asked question about your work?**

The first on the list is: “*Why is it different to perform surgery in patients with a bleeding disorder?*” Usually, orthopaedic surgeons not involved in haemophilia care think that it is the same to perform surgery on a person with haemophilia after adequate replacement therapy. Unfortunately that is false, because in general replacement surgery is not common in young patients and our patients have multiple target joints and comorbidities. So, an orthopaedic surgeon working in this field needs a very open mind to take in account different variables, which may influence the final outcome of the surgery.

7. **What is the next big thing that is coming in your field of work?**

Although I’m an orthopaedic surgeon, I will not answer a ‘new joint replacement’ or a ‘new method to treat cartilage damage.’ Instead I think that all of our community should be focused on the method of early diagnosis and prevention of joint damage. Hence, the next big thing would be the availability of more sensitive tools of diagnosis (i.e. ultrasound, gait analysis) as described above and the improvement of knowledge on the pathogenesis of arthropathy. This is especially true now that more and more children have access to prophylaxis and are becoming increasingly active.

8. **If you had not been working on this topic, you would have been working on...?**

When I was younger I wanted to become a football player (we have a healthy national obsession with football) but I quickly realised that this will not be possible and so I thought that if I became a sports doctor and focus on sports traumatology, it would be one way to get closer to professional football. So, I would have to say sport traumatology.
9. **What would be your advice or recommendation to someone with a bleeding disorder?**

My recommendation to someone with a bleeding disorder would be to do regular exercise in order to maintain muscles and joints active and if possible to play sports by choosing those more adequate for their individual body structure. Even among healthy individuals not all sports fit everyone so it is essential to tailor the haematological treatment and sport activity to each patients’ profile.

I am often asked by my younger patients if they can play football and traditionally this is not a recommended sport for people with bleeding disorders because of the high impact on the joints and potential physical contact. Nonetheless I say yes on the one condition that if there is any problem, my patients tell me immediately. I always tell my patients that I dreamed of becoming a football player but ended up working as doctor, which in my opinion is not such a bad thing after all. There are other options for physical activity such as swimming, rowing, cycling. So my second advice to patients would be to have an open and honest relationship with their physician.

10. **What is one question you have always wanted to ask to someone with a bleeding disorder but have never dared to ask?**

As just mentioned I have a very open relationship with my patients and we talk about all sorts of topics and therefore there is no one question that I have never dared to ask.

* Dr Gianluigi Pasta is an orthopaedic surgeon at the Orthopaedic and Traumatology Department, Fondazione IRCCS Ca' Granda, Ospedale Maggiore Policlinico, Milan, Italy.

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

Checkmate on haemophilia: identifying the real cost of haemophilia with the CHESS study

* Jamie O’Hara interviewed by Laura Savini**

1,285 patients and 139 haematologists from five different countries have taken part in the largest ever study into the socio-economic costs associated with severe haemophilia A and B – and the results will soon be published. Jamie O’Hara tells us more.

When Jamie O’Hara joined the Board of Trustees of the Haemophilia Society in the United Kingdom (UK), he was asked to help with the communication about health economics in relation to the cost of haemophilia care. Jamie is a health economist by training and profession and has his own health economics consultancy firm. He is also a lecturer at the University of Chester in the UK. For all of these reasons, the UK Haemophilia Society identified him as the ideal candidate for this particular task. However, Jamie soon realised that there was no hard scientific evidence about the costs related to haemophilia care such as formal and informal care and the economic impact on patients’ everyday lives. The sole information available was anecdotal with no economic model to analyse it. So armed with his knowledge and skillset and the support of the UK Haemophilia Society, he started a study on the topic.

Jamie O’Hara had worked on similar studies in other disease areas and suggested to the Haemophilia Society UK to start their own study on the real cost of haemophilia. The Society agreed and in partnership with the University of Chester and funding from pharmaceutical companies, the study started.
A cross-sectional investigation was conducted taking cross-sectional samples from the five biggest countries in the European Union. Concretely this meant that 139 haematologists from across Europe’s five biggest countries were recruited and through them the study’s investigators were able to reach out to almost 1,285 patients to look at every aspect that haemophilia care could cost society. To be included in the study, patients had to be adult males with severe haemophilia A or B. The study started by looking firstly at the costs that could be extrapolated from physicians such as treatment strategies, consultation frequency, hospitalisation, joint replacements, arthroscopies, etc. Through this first stage, investigators were able to collect around 100 different variables on each patient. The second step involved reaching out to those patients individually and asking them to fill in an additional questionnaire to collect information about informal care, health-related quality of life, lost days at work, adherence, etc. Patients were also asked to give out information about their treatment to validate the information given by physicians.

The full report of the study will not be put into the public domain, however, a series of publications to look at the findings in different areas of the study will be published. A first manuscript has been submitted to the scientific journal *Haemophilia* and is awaiting peer review. Currently, Jamie is in the process of writing the second manuscript for publication, which focuses on target joints. He is also planning an additional three such publications.

The study has attracted a lot of interest and there are some talks of expanding the survey to other geographical areas such North America and Eastern Europe as well as other medical areas such as paediatrics.

In Jamie’s opinion, there will be an increase in scrutiny from payers with regards to the cost of haemophilia treatment. This will be particularly true with the advent of extended half-life products. Claims for added-value of a pharmaceutical product and treatment protocols will certainly be continued to be looked over by payers who are under increasing pressure to contain healthcare costs. Therefore it is important to arm patient organisations with the right advocacy data and arguments to make the case to receive the most efficacious treatment. This situation was recently highlighted by the rapid report on haemophilia care from the Institut für Qualität und Wirtschaftlichkeit im Gesundheitswesen (IQWiG) (Institute for Quality and Economics in the Health Care System) in which on-demand treatment is viewed at the same level of effectiveness as prophylaxis. “As patients, we well know and can see, by simply looking at two patients from different generations, that prophylactic treatment makes a huge improvement in the patients’ quality of life in terms of reduction of bleeds, preservation of healthy joints and being able to lead physical activity, which is very beneficial to maintain joints health. Everyone knows this but there is no hard evidence about it, so it is important to start developing evidence,” states Jamie.

The next steps are for a CHESS Steering Committee to oversee the finalising of the planned publications but also to direct the work beyond the EU five. A CHESS working group will also be set up to provide advice to the Data and Economics Committee of the European Haemophilia Consortium (EHC) on how to best use the data for advocacy. The working group will also support EHC National Member Organisations and give them guidance on how to use the CHESS data for advocacy.

* Jamie O’Hara is a member of the EHC Data and Economics Committee. He will speak about the results of the CHESS study during the 2016 EHC Annual Conference in Stavanger.

** Laura Savini is the EHC Communications and Public Policy Officer.
Living with haemophilia: stories from five European Countries. A documentary film by Goran Kapetanovic

Goran Kapetanovic* interviewed by Laura Savini**

Participants during the EHC Workshop on New Technologies in Haemophilia Care (see pg 30) were invited to preview excerpts from a new documentary film following five individuals living with haemophilia in Europe. The movie was produced and directed by Mr Goran Kapetanovic, an award-winning Swedish director of Bosnian origin.

Goran was first commissioned to develop a short movie on haemophilia a little over a year ago – a project that has been funded by an educational grant from Sobi. The aim of the project was to develop a short movie that would quickly explain what haemophilia is and how it impacts the daily lives of the individuals affected by it. Little did Goran know that what was meant to be a short assignment would turn into a year-long project to develop a full hour documentary that would bring him to meet with several individuals located across Europe. Goran describes the experience as very enriching, having allowed him to meet some extraordinary individuals with tremendous character and perseverance in coping with the fallouts of the disease.

Prior to starting the project, Goran had some idea of what haemophilia was, seeing he has friends who are impacted by the disease. However, this year-long project really allowed him to further develop his knowledge and to fully explore the impact of the condition. Goran accepted to carry out the project on the one condition that he could have full artistic freedom. So, he started to look for candidates to film. The European Haemophilia Consortium (EHC) put him in touch with some of its National Member Organisations (NMOs) and more specifically with France, the United Kingdom, Sweden, Bulgaria and Romania. It was important for Goran that the film be representative of different types of patients from Eastern, Central and Western Europe as well as different age groups, including younger and older patients in the movie. Goran also met physicians like Prof Flora Peyvandi from the University of Milan, Italy and a member of the EHC Medical Advisory Group and Dr Andreas Tiede from the Haemophilia Centre in Hannover, Germany. Goran also interviewed Mr Brian O’Mahony, EHC President.

Goran’s primary objective was to tell the personal story of individuals affected by haemophilia without having haemophilia predefining how he would approach his subjects. With this approach, the viewer first meets the individuals and through their testimonials learns more about haemophilia and discovers how the disease impacts them in their daily lives. The documentary film, which is still being edited into a final product, shows in a series of snapshots into the lives of these individuals. The viewer follows them around while they are carrying out daily activities and we hear testimonials from them and their families about their lives. The result is a beautifully shot film that is both personal and touching. We are brought to meet these individuals in a very natural way and we, as viewers, are invited to witness

Goran learned a lot from this experience. One of his main takeaways is that even with good access to treatment, haemophilia still has a significant psychological impact on patients and families alike.

President.

Goran Kapetanovic directed a documentary on living in Europe with haemophilia (photo courtesy of Goran Kapetanovic)
‘regular’ patients (i.e. not trained patient advocates) giving very candid accounts of their lives. It is through the lives of these individuals that we learn more about haemophilia and topics such as the historic progression of the treatment and what it meant to live without treatment; to first receiving coagulation factors; the contamination tragedy; living with inhibitors; living with moderate haemophilia; being affected by joint bleeds; and being a young father with haemophilia.

For Goran, it was important to gain the confidence of the individuals he interviewed so that he could portray them in a fair, intimate and respectful way without being intrusive. Goran explained that developing a documentary like this one was a process and that it was important that the people he interviewed were able to familiarise themselves with him and to feel like they could relax and act normally around him.

Goran learned a lot from this experience. One of his main takeaways is that even with good access to treatment, haemophilia still has a significant psychological impact on patients and families alike. However, he also stressed the strength of people with haemophilia, their love for life and their eagerness to share their story, for which he was very grateful.

The final documentary film is currently being edited and will be broadcasted in early 2016. A website with the individual stories of the individuals from the documentary is being developed and will be accessible to all in the new year. Some of the EHC National Member Organisations (NMOs) have shown interest in trying to have the movie broadcasted on their national television stations in time for World Haemophilia Day on 17 April. Some NMOs have also offered to dub or subtitle the movie. If you or your NMO would like to contribute to the diffusion or translation of this documentary film, please get in touch with Laura Savini at laura.savini@ehc.eu.

*Goran Kapetanovic is a film director born in Sarajevo in 1974. He studied to become a director at the Dramatika Institutet de Stockholm, Sweden, and also studied History of Art at the University of Lund in Sweden. His movies have received many international prizes and recognition including his movie Kiruna-Kigali was shortlisted for the Oscars.

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

Making Treatment for All a reality: The WFH Humanitarian Aid Programme expands to provide essential treatment for those in need

By Assad Haffar, Humanitarian Aid Director, World Federation of Hemophilia (WFH)

It is hard to imagine the reality of families with no access to treatment and care for their sons and daughters. Often medical professionals in developing countries try their best with the limited resources that they do possess. Access to a limited supply of treatment products is typically only available in major city centres and families often have to travel by whatever transportation is possible over long distances. For a family with a young child suffering from a bleed, this journey can be exhausting at the best of times but most likely terrifying.

For the World Federation of Hemophilia (WFH) its vision of “Treatment for All” means that one day, all people with a bleeding disorder will have proper care, no matter where they live. This includes access to proper diagnosis, management, and care by a multidisciplinary team of trained specialists. It also means sustained and predictable access to safe and effective treatment products for all people with inherited bleeding disorders.

Assad Haffar is the WFH Humanitarian Aid Director (photo courtesy of WFH)
This is a monumental task, especially considering that a majority of people living with a bleeding disorder still receive very inadequate care or no treatment at all. Most of them live in countries where there is limited access to diagnosis and treatment and due to this lack of treatment people with severe haemophilia in these countries often do not survive to adulthood. If they do survive, they face a life with severe disability, isolation, chronic pain, and very often early death. The reality is that a majority of people with haemophilia in developing countries die before age 20.

The greatest challenge to achieving the WFH’s vision of Treatment for All is how to address this lack of access to diagnosis, care and treatment for the most vulnerable in the global bleeding disorders family. It is almost inconceivable to those living in developed countries that both children and young adults can face these kinds of hardships. However, in most parts of the developing world, governments do not have the financial resources to provide treatment products at the current prices for their bleeding disorders populations. Therefore, the need for a sustainable and predictable humanitarian aid stream is the only chance for these groups of patients and their families to receive access to diagnosis and then treatment.

Created in 1996, the WFH Humanitarian Aid Programme channels donations of life-saving treatment products to people with bleeding disorders who need them all around the world. This programme is one of the many ways in which the organisation supports its vision of Treatment for All. As of today, the WFH Humanitarian Aid Programme has distributed 266 million International Units (IUs) to 87 countries, helping some 90,000 people with bleeding disorders directly who are in urgent need. In 2015, the WFH sent 4.5 million IUs of donated treatment products within Europe to Armenia, Kyrgyzstan, Moldova, Ukraine and Uzbekistan. The WFH is committed to continuing to provide support for countries most in need, including those within Europe.

One of the WFH’s key strategic goals for the next three years is to make more donated products available in developing countries, which will in turn make humanitarian aid more predictable, and care, sustainable. The WFH Humanitarian Aid Programme incorporates a range of integrated care development training programmes to ensure the local infrastructure and medical expertise are available to optimize and appropriately use donated products. With multi-year donations and a steady flow of treatment product to the WFH network, it will also be possible for people with bleeding disorders in the developing world to have access to treatment for emergency situations, acute bleeds, corrective surgeries and also prophylaxis for young children.

In 2015, the WFH Humanitarian Aid Programme expanded to include 500 million IUs pledged by Biogen and Sobi over the next five years. There have been other major commitments to providing a predictable and sustainable donation supply by the continued efforts of the Canadian Blood Services, Biotest, and Grifols with Project Recovery, and the work by the Italian National Blood Services through Project Wish. In addition, CSL Behring and Grifols have signed multi-year commitments of treatment products, which will contribute to the expansion of the WFH Humanitarian Aid Programme. Through this commitment and support there will be a substantial impact on the breadth and scope of care around the world.

In mid-October, the WFH led a delegation to Senegal to mark the expansion of the WFH Humanitarian Aid Programme. Patients and their families came from across Senegal to meet with the delegation and tell their stories. Each of these families has their own history of their struggles to secure a diagnosis, let alone treatment. Their stories mirror the experiences for many in developing countries and they continue to underline the absolute importance of providing treatment for all people with inherited bleeding disorders. Treatment for All is truly the responsibility of all.
News from our corporate partners

Baxter’s BioScience becomes Baxalta

Commitment to the haemophilia community is our priority

Baxalta Incorporated was launched on July 1st, 2015, as a new, publicly traded global biopharmaceutical leader advancing innovative therapies that improve the lives of patients with rare diseases and underserved conditions. Baxalta was formerly the BioScience business of Baxter International Inc, and thus comes from a rich legacy of innovation and community partnership in haemophilia and immunological conditions. Baxalta will continue to focus and expand on this heritage, whilst also building a specialty in oncology, focusing on rare cancers with limited treatment options.

The company is strongly committed to patient-focused innovation, with USD 595MM committed to Research & Development in 2014, 20 product launches targeted by 2020, 40 programmes in pipeline (including 13 in Phase III+) and a Global R&D Innovation Centre in Cambridge, Massachusetts. Seven key product approvals have taken place in the last 24 months, and 12 acquisitions and key strategic partnerships have been negotiated. The company operates in 100 countries, and has eleven high quality manufacturing sites in seven countries.

At Baxalta, patient-centricity is at the core of the company philosophy. We strive for continued pursuit of innovation and commitment to those living with the rare conditions we support, and our aim is to engage communities to ensure their voice is embedded across the company.

Community partnership with the Haemophilia & Blood Disorders Community

Baxalta is committed to carrying on the legacy of Baxter’s long-standing partnership with the European haemophilia community – both with national haemophilia societies and at European level. In 2004, Baxter led the industry in its support of the European Haemophilia Consortium (EHC). It was also the first company to support the European Association for Haemophilia & Allied Disorders (EAHAD) in 2008. It continues to support both of these organisations through programmes that reflect the needs of the European haemophilia community.

One such programme, launched in 2015, is the EHC’s European Inhibitor Network (EIN). Through our support and in partnership with the haemophilia community, Baxalta wants to ensure that the needs of those living with inhibitors are prioritised and supported. In addition to the EIN, Baxalta also supports the EHC’s Youth Workshops, Policy Roundtables and Annual Congress. In 2015 Baxalta, committed to support the EHC’s New Technology Workshop, as well as the pilot of the new EHC Leadership Meeting which took place in Belgrade in October. Baxalta looks forward to building on this legacy of partnership and support going forward.

Our vision for a Life Without Bleeds

Baxter’s BioScience, now Baxalta, has strived to serve the haemophilia community by continually innovating over 60 years and developing new and improved treatments for those living with this lifelong condition. It was the first company to develop commercially produced FVIII concentrate in 1968; it then pioneered treatment for those with inhibitors in 1977; made available the first recombinant treatment in 1992; followed by the first recombinant treatment made without the addition of human or animal proteins in 2003.
Our vision for the future and what we strive for every day is a life without bleeds, one patient at a time. To us, this means a world where people living with haemophilia can choose to live their lives because they are healthy and bleed-free. We question any approach that accepts bleeds as ‘part of life’, and instead actively work to prevent bleeds in every person with haemophilia through effective prophylaxis and personalised treatment, giving those with haemophilia the exact amount of direct factor replacement at the exact dose and frequency to give them the best chance to live without bleeds.

As part of this vision, Baxalta continues its pioneering journey, and has a rich haemophilia treatment pipeline – with new recombinant treatments available for Haemophilia B and Acquired Haemophilia A. Looking to the future, Baxalta also has a new recombinant treatment under development for those with inhibitors and two gene therapy products in development for Haemophilia A and B, as well as extended half-life treatments for Haemophilia A.

**New treatments for Rare Blood Disorders**

With this legacy in Haemophilia, Baxalta is also looking to expand its support for other rare blood disorder communities. Its growing haematology pipeline includes early stage development of a recombinant treatment for von Willebrand Disease in addition to new treatments for Sickle Cell Disease and hereditary Thrombotic Thrombocytopenic Purpura (hTTP).

**Renewing our commitment to the community**

As we progress together in this new era of innovation, Baxalta renews its commitment to supporting the patient community, which has done a tremendous amount to advance standards of care for those living with haemophilia. As Baxalta, we want to continue to be a trusted and valued industry partner, striving to ensure that the voice of those living with haemophilia and blood disorders is strong and influential across all aspects of treatment and care decision making.
EHC 2015 Newsletter

At Pfizer Haemophilia, we’re proud of our heritage of innovation through collaboration and partnership in haemophilia care. In 2015, we further cemented our long-standing commitment to the community through the expansion of established programmes, and the launch of exciting new initiatives for researchers, physicians, and people living with haemophilia, globally.

Miles for Haemophilia: Your Personal Best

In 2015, we celebrated 1 year of our celebrated Miles for Haemophilia disease awareness campaign, with the launch of Your Personal Best. This important campaign has been running across Europe and at International Congresses with the aim of raising awareness of the importance for people with haemophilia of staying active to maintain healthy joints.

Through a partnership with Alex Dowsett, a professional cyclist with severe haemophilia A, we encouraged people to pledge their personal best to support the haemophilia community. So far, the campaign has received pledges of over 70,000 Km, from 10,000+ participants, resulting in over €54,000 donated to local haemophilia societies.

We are proud that through our work with the Miles for Haemophilia: Your Personal Best campaign, we have been able to partner with local and regional patient organisations globally to highlight to the haemophilia community that with the appropriate care, management, treatment and attitude, anyone with haemophilia can lead a fulfilled life.

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

Pfizer Ultrasound Programme

Point-of-care ultrasound is a promising technique for routine clinical assessment of joints in people with haemophilia, and can be used by non-imaging, haemophilia specialists to detect early signs of damage during routine clinical assessment.

Haemophilia Early Arthropathy Detection with UltraSound (HEAD-US) has been developed by a Radiologist, Professor Carlo Martinoli (Genoa, Italy) with the aim of integrating point-of-care ultrasound into routine clinical practice in haemophilia centres as a close complement to physical examination.

Pfizer Haemophilia is proud to support a scientific steering committee in expanding our knowledge and understanding of the value of point-of-care ultrasound through global, networked collaborations. In 2015, the results of a key study in the validation of this tool were presented at the ISTH congress in 2015. Pfizer Haemophilia is committed to working globally to support the study of this point-of-care tool, in addition to investing in training haemophilia treaters and physiotherapists in the technique in order to bring this innovation in imaging direct to patients.
Haemoassist® 2 Patient App

Haemoassist® 2 is an intuitive, dynamic alternative to patient’s current diary system, allowing real-time reporting and documentation at the touch of a button. The award-winning Haemoassist® 2 system has a range of core features to benefit patients, physicians, and the administrative burden associated with patient data collection.

In 2015, Pfizer Haemophilia were proud to support / fund the launch of the Haemoassist® 2 tool in a number of new countries across Europe, to support people with haemophilia regardless of their choice of clotting factor concentrate.

There’s only one way to succeed: together

At Pfizer Haemophilia, we understand the value of collaboration and partnership.

Since 2001, Pfizer Haemophilia has been the exclusive sponsor of the WFH Twinning programme, an initiative that aims to help emerging haemophilia treatment centres develop partnerships with well-established, knowledgeable, and experienced centres. The treatment centre twinning programme also benefits established centres by giving them the opportunity to share their expertise and make a global difference.

In collaborating with advocacy groups, such as EHC, we build a collective body of expertise and shared resources that can help meet the spectrum of community-needs along the disease journey.

For the past 3 years Pfizer has partnered with EHC to provide support for targeted economics and Health Technology Assessment (HTA) training to patient representatives from Western, Central and Eastern Europe. These workshops are critical in ensuring that National Member Organisations (NMOs) have a high-level, appropriate and sophisticated understanding of the economic issues and how to tackle them with their national payers in order to ensure continued patient access based on safety, efficacy and choice, in addition to cost.

Collaborations with Biotech and academic institutions bring specialised, deep expertise that speeds new discovery of potential treatments. For example, our collaboration with the UKs Global Medical Excellence Cluster (GMEC) under the Rare Disease Consortium (RDC) is building a framework for gene therapy.

Our unique partnerships extend and further evolve the collaborative model. Together we are gaining greater knowledge faster, eliminating unproductive avenues of exploration sooner, and testing and improving potential new therapies more efficiently.

We look forward to continuing to support and develop further these important collaborations and partnerships, in the shared pursuit of our Pfizer Haemophilia commitment to Change the World for People with Haemophilia.
Second Youth Debate held during EHC Annual Conference

For the second year, Sobi donated its company symposium at the EHC Conference to the European Haemophilia Consortium (EHC) to help support its youth leadership training work, in order to hold a debate together with the more senior leaders in the haemophilia community. Developing the leaders of the future is a key strategic focus for the EHC; the concept is that having this opportunity to work with the more senior leaders helps the young people develop key skills such as advocacy, public speaking and advancing arguments around important issues in haemophilia care as part of the overall work the EHC does on youth leadership training.

The young debaters were nominated from the pool of participants of the Youth Leadership Workshop that was organised by the EHC for their membership in July 2015, where younger members of the EHC with high potential and a keen interest in taking a more prominent role in representing the haemophilia community, came to develop their skills. They were then paired up with members of the EHC’s Medical Advisory Group (MAG) to prepare a debate during the annual EHC Conference, this year held from 2-4 October 2015 in Belgrade, Serbia.

The symposium, held on the evening of Friday 2 October 2015, was the moment for the debate to happen. The young debaters were asked to advance arguments on topics of importance to the community, as diverse as tender-based procurement systems, treatment of patients with inhibitors and whether women with bleeding disorders are neglected by healthcare systems and the treating community. To make it more challenging, the MAG members were asked to argue the “other” side of the proposed statement, meaning that they had to play “devil’s advocate” and defend the “Status Quo” versus the “one Direction of the youth debaters.

Introducing the debates humorously as “a legal blood sport,” Brian O’Mahony, EHC President and in his role as Chair of the debate, set a strict time limit for all speakers. Although this year he had replaced his terrifyingly loud bell with a much more restrained – but nevertheless effective – xylophone chime.

The speakers argued passionately for their sides, presenting strong evidence as well as using classic debating tricks – as Brian O’Mahony said “it is always a good idea to quote approvingly from a paper published by the Chairman.”

Professor Paul Giangrande revelled in the role of devil’s advocate, risking the wrath of women everywhere to argue that women with bleeding disorders are not neglected and pointing out that even though the EHC was “an organisation set up by men with haemophilia for men with haemophilia” one of the two conference workshops and one of the plenary sessions focused on women with bleeding disorders. Despite the raucous laughter Professor Giangrande did not manage to convince everyone that women with bleeding disorders do not have real cause for concern given the risk to women facing misdiagnosis and a lack of clinical expertise, even in some haemophilia centres.

The youth debating team. From left to right: Prof Mike Makris, Mr Ilmar Kruis, Mr Brian O’Mahony - moderator of the debate - Prof Flora Peyvandi, Mr Stefan Radovanovic and Mr Ivan Sebest. Prof Paul Giangrande also on the debating team is not in the picture.

The youth debating team. From left to right: Prof Mike Makris, Mr Ilmar Kruis, Mr Brian O’Mahony - moderator of the debate - Prof Flora Peyvandi, Mr Stefan Radovanovic and Mr Ivan Sebest. Prof Paul Giangrande also on the debating team is not in the picture.
The topics were serious, the debates were serious and the challenges faced by the community were brought to light in a series of well-prepared and extremely well-argued positions. The debates played out in front of a packed room and increasingly lively atmosphere; and at the end, votes from the audience pronounced two of the three debates a draw, and in the last debate declared the youth team’s victory, convincing the audience and participants that people with inhibitors are, indeed, neglected by healthcare systems.

After the formal debates, the MAG members exchanged viewpoints on a variety of aspects of the subjects of the debate, in response to questions from the floor. How to create a good tender process? It is clear that the tenders are only as good as the people in the room taking the decision, said Brian O’Mahony, based on his extensive experience. Having clear and robust criteria, which cover price but are not limited to that, is also important. If tenders are organised, the money saved on procuring cheaper factor concentrates should be used for the benefit of the community. What is a fair price for a product and how to value and reward innovation?

So another key question is: how effective is the youth debate at developing younger leaders? It is clear that the senior leaders take great pride in preparing their partner in the debate, even if they prepare them so well that it means that they might have to lose the debate to the young leader as a result. But that just shows what a great job they have done in passing on their skills and knowledge and how serious the young debaters are about taking up their role of representing the community; and Professor Peyvandi seemed absolutely delighted that her protégé in the debate on inhibitors was voted the winner in that particular interchange.

It appears that this debate is well-set to become an EHC Conference tradition, and Brian O’Mahony closed the meeting thanking all the participants for their excellent – and stimulating – contributions and inviting participants to join again next year in Norway. We look forward to it!
Bayer’s Commitment to the Worldwide Hemophilia Advocacy Community

Bayer is dedicated to supporting the next generation of advocates by sponsoring HemophiliaLEAD, a leadership and advocacy development program open to people around the world who want to be more involved in the hemophilia community.

Bayer developed the HemophiliaLEAD program based on the insight that providing skill-building opportunities is an effective way to engage and support advocacy leaders, from youth who have an interest in supporting advocacy efforts in their communities to seasoned professionals. HemophiliaLEAD equips its members with the skills and knowledge to help improve the lives of those living with hemophilia. The program includes four components:

Step Up Reach Out (SURO) is an international youth advocacy program designed to develop young adults ages 18-25 diagnosed with hemophilia A or B who have the drive to become leaders.

Adult Fellowship for Integrating Responsible Mentors (AFFIRM) is a two-year international fellowship program designed to help men aged 26-38 advance their advocacy and leadership skills.

The Hemophilia Advocacy Advisors Board (HAAB) brings together top patient advocates from around the world to identify and address unmet needs in ways that enable access to optimal, sustainable care.

The Global Hemophilia Advocacy Leadership Summit is an annual event organized by the HAAB that convenes leading global advocates, expert stakeholders and opinion leaders to discuss topics of importance to the hemophilia community.

Through HemophiliaLEAD, Bayer has contributed to the growth of more than 200 individuals from 40 countries. The impact of this program can be seen in the global hemophilia communities, where program alumni are taking leadership positions, such as Lino Hostetler, 2009-2010 SURO graduate, 2012-2014 AFFIRM graduate, and current president of the Swiss Hemophilia Society. “The SURO program changed my life. Since graduating, I have become involved with the global community by attending European and WFH World Congresses, and on a local level I am getting others engaged through my National Member Organization. The global network among men with hemophilia has grown immensely since the introduction of the SURO and AFFIRM programs.”

With HemophiliaLEAD by Bayer, patient need inspires every action, whether it is research, support or treatment of hemophilia. Through unique patient-support groups, Bayer is dedicated to the hemophilia community and will continue to deliver on its promise of superior patient care through innovation and expanded treatment options. For more information, please visit www.hemophiliaLEAD.net.
CSL Behring: Driven by Its Promise to Patients

It began with a promise. More than 100 years ago, CSL Behring was formed to save lives using the latest technologies. This promise led to the first Nobel Prize in Medicine, which was awarded to Emil von Behring for his work on serum therapies.

Today, CSL Behring has grown into a global business with one of the world’s largest portfolios of bleeding disorders therapies for patients. These therapies treat haemophilia A and B, von Willebrand disease and a number of other rare bleeding disorders including congenital fibrinogen deficiency and factor XIII deficiency.

CSL Behring focuses its world-class research and development, high-quality manufacturing, and patient-centred management to develop and deliver innovative biotherapies and support programmes that improve patient well-being. This is evidenced by CSL Behring’s advancements in its novel recombinant factor development programme that strives to improve treatment for people with haemophilia.

Moreover, CSL Behring continues to make substantial investments in state-of-the-art facilities for production of its recombinant proteins in Marburg (Germany), Lengnau (Switzerland), Broadmeadows (Australia) and Parkville (Australia).

These advancements and investments further demonstrate CSL Behring’s drive to care for patients and deliver on its promises to the global bleeding disorders community. For more information about CSL Behring, please visit [www.cslbehring.com](http://www.cslbehring.com) or follow the company at [www.twitter.com/cslbehring](http://www.twitter.com/cslbehring).
innovation takes action

In the face of limited access to care and treatment in the developing world, brave people are dedicated to enacting change. The Novo Nordisk Haemophilia Foundation (NNHF) partners with these people to provide the funding and support they need to succeed in improving access to better care. Together, the foundation and its partners have overcome tremendous obstacles for the benefit of people with haemophilia and allied bleeding disorders. Country by country, patient by patient, the foundation is taking an innovative approach to helping make sustainable change.

The foundation’s initiatives have resulted in training for 23,700 healthcare professionals, diagnoses for 18,400 people with haemophilia, and educational activities reaching 26,200 people worldwide. In China, Venezuela, Pakistan, and dozens more countries, the foundation is proud to be a part of the ground-breaking work that is delivering life-changing impact.

Find out more about the Novo Nordisk Haemophilia Foundation at nnhf.org and how Novo Nordisk is changing possibilities in haemophilia at changingpossibilities.com
BIOTEST AG supports 'Project Recovery,' turning unused blood products from Canadian blood donations into hemophilia medicine for developing countries – an update

BIOTEST AG, together with the World Federation of Hemophilia (WFH), Canadian Blood Services (CBS) and Grifols, has entered 2013 into an agreement called Project Recovery, which will transform previously discarded cryoprecipitate from Canadian blood donors into BIOTEST’s factor VIII concentrate.

This was the first time anywhere in the world that such a partnership has been created, transforming surplus cryoprecipitate into FVIII for humanitarian use. With Project Recovery, the cryoprecipitate will be harvested by Grifols at its plant in the United States (US) and then transported by BIOTEST AG to Germany for manufacturing.

Project Recovery is now running for two years and so far BIOTEST AG donated 2.98 million IU of factor VIII in the name of CBS. Another 2 million International Units (IUs) are available for immediate call off by the WFH. BIOTEST AG will make 2.3 million IUs of factor VIII more available in the near future.

According to Alain Weill, WFH President, "Project Recovery has a potential to improve the lives of thousands of people with haemophilia all over the world. It allows WFH to carefully plan where and when these essential medicines will be distributed and thereby the benefits of this wonderful humanitarian endeavour."

This agreement is an important milestone for BIOTEST’s approach to improve the haemophilia care worldwide.

uPatient & uClinics Hemophilia, new digital solution to optimize patient treatment & management

uPatient Hemophilia: An easy web based diary to follow-up patient’s infusions, bleeds, appointments... All presented as an intuitive platform aimed at improving adherence to treatment for people living with hemophilia A, B and von Willebrand Disease.

uClinics Hemophilia, on the other side, facilitates the healthcare provider to interact with patients and enables effective disease management due to real-time information and alerts.

uPatient Hemophilia and uClinics Hemophilia, are available worldwide for free in four different languages, led by Medtep, supported by GRIFOLS and were launched in the World Congress of the World Federation of Hemophilia (WFH) in Melbourne in 2014. “This is easy to use and I see how it will help me improve my adherence to the treatment”, said M.G, a 41-year-old male Hemophilia A patient, after using the platform for the first time.

Medtep, a digital health company with an extensive background in rare and chronic diseases, is now committed to make an impact in hemophilia. Thanks to this, Medtep has started a clinical study aiming...
to prove the increase in treatment adherence and the improvement of the patient’s quality of life using the platform.

Visit [www.hemophilia.upatient.com](http://www.hemophilia.upatient.com) & [www.hemophilia.uclinics.com](http://www.hemophilia.uclinics.com) and discover all this new platforms can offer you!

**Announcements**

**EHC Calendar of Events**

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<tr>
<th>Date</th>
<th>Event Description</th>
<th>Location</th>
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<tbody>
<tr>
<td>Feb 16</td>
<td>EHC Round Table on Inhibitors – Open to NMOs and selected participants</td>
<td>Brussels, Belgium</td>
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<tr>
<td>Apr 8-10</td>
<td>EHC Youth Leadership Conference – Open to NMO only</td>
<td>Location to be announced</td>
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<tr>
<td>Apr 20</td>
<td>World Haemophilia Day Celebration – Open to all</td>
<td>Brussels, Belgium</td>
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<tr>
<td>Apr 20</td>
<td>EHC Round Table on Ageing and Haemophilia (TBC) – Open to NMOs only and selected participants</td>
<td>Brussels, Belgium</td>
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<tr>
<td>Jun 10-12</td>
<td>2nd EHC Leadership Conference – Open to NMOs only</td>
<td>Brussels, Belgium</td>
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<tr>
<td>Jun 14</td>
<td>EHC Round Table on HCV (TBC) – Open to NMOs only and selected participants</td>
<td>Brussels, Belgium</td>
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<tr>
<td>Sep 9-11</td>
<td>EHC Workshop on Tenders and Procurement – Open to NMOs only</td>
<td>Location TBD</td>
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<tr>
<td>Oct 7-9</td>
<td>EHC Annual Conference – Open to all</td>
<td>Stavanger, Norway</td>
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<tr>
<td>Nov 18-20</td>
<td>EHC Workshop on New Technologies in Haemophilia Care – Open to NMOs only</td>
<td>Location TBD</td>
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<tr>
<td>Nov 28</td>
<td>EHC Round Table on personalised outcome measures</td>
<td>Brussels, Belgium</td>
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<tr>
<td>Dec 1-4</td>
<td>Inhibitor Summit</td>
<td>Location TBD</td>
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*To find out more about EHC events visit [http://www.ehc.eu/calendar-of-events/events/](http://www.ehc.eu/calendar-of-events/events/)*

**Other Events**

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<tr>
<th>Date</th>
<th>Event Description</th>
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<tr>
<td>Mar 22-23</td>
<td>Annual General Assembly of the European Patient Forum (EFP)</td>
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Mar 22-23  International Plasma Protein Congress (IPPC)  

Apr 13-17  International Liver Congress  

Apr 17-19  13th International Hemophilia Congress of Turkey  
Instanbul, Turkey – More information at http://www.turkiyehemofilikongresi.com/

May 6-7  Wild Bad Kreuth Initiative IV Symposium  
Freising, Germany

May 25-26  IPFA/PEI 23rd International Workshop on Surveillance and Screening of Blood Borne Pathogens  
Lisbon, Portugal – More information at http://bit.ly/1IQh19s

May 25-28  62nd Annual Meeting of the Scientific and Standardisation Committee of the International Society for Thrombosis and Haemostasis  

May 26-28  8th European Conference of Rare Diseases and Orphan Products (ECRD)  
Edinburgh, United Kingdom – More information at http://www.rare-diseases.eu/

Jul 24-28  2016 World Congress of the World Federation of Hemophilia (WFH)  

Publications

Positive News

Positive News is a bi-annual magazine produced by the Irish Haemophilia Society (IHS) that covers issues surrounding hepatitis C and haemophilia such as treatment advances, treatment protocols and so on. The latest edition of this magazine was published in December and includes topics such as:

- Patient experiences and perspectives with new HCV treatments
- An update from the AASLD Liver Meeting in November 2015
- Difficult to treat groups of people
- Ribavirin and its role in new HCV treatments
- A global review

The magazine can be consulted freely and online on the IHS website. For further information and to register on the distribution list, please contact Declan Noone at declan@haemophilia.ie
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<tr>
<th>Time</th>
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<tr>
<td>08.30</td>
<td><strong>General Assembly</strong> (NMOs only)</td>
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<tr>
<td>10.00</td>
<td>Tea/Coffee – Exhibition Area – iPad raffle</td>
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<tr>
<td>10.30</td>
<td><strong>General Assembly</strong> (NMOs only)</td>
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<td>12.30</td>
<td><strong>Buffet Lunch</strong></td>
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<td>13.30</td>
<td><strong>Organisation of Haemophilia Care in Norway</strong></td>
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<td>Chair: Pål Andre Holme, Oslo University Hospital</td>
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<tr>
<td>13.30</td>
<td>Delivery of haemophilia care in Norway</td>
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<td></td>
<td><em>Pål Andre Holme, Oslo University Hospital</em></td>
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<tr>
<td>14.00</td>
<td>Delivery of haemophilia care in other Nordic countries</td>
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<td><em>Jan Astermark, Lund University Hospital</em></td>
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<td>14.30</td>
<td><strong>Industry symposia 1</strong></td>
</tr>
<tr>
<td>16.00</td>
<td>Tea/Coffee – Exhibition Area</td>
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<tr>
<td>16.30</td>
<td><strong>Industry symposium 2</strong></td>
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<tr>
<td>18.00</td>
<td><strong>Updates on dental care and von Willebrand treatment</strong></td>
</tr>
<tr>
<td></td>
<td>Chair: Flora Peyvandi, University of Milan</td>
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<tr>
<td>18.00</td>
<td>Update on von Willebrand Treatment</td>
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<tr>
<td></td>
<td><em>Mike Laffan, Imperial College London</em></td>
</tr>
<tr>
<td>18.30</td>
<td>Dental care</td>
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<tr>
<td></td>
<td><em>Alison Dougall, Trinity College Dublin</em></td>
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<tr>
<td>19.30</td>
<td><strong>Welcome Reception and Buffet Supper</strong></td>
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<td></td>
<td><em>Helene Døscher, President, Norwegian NMO</em></td>
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<td></td>
<td><em>Brian O’Mahony, President, EHC</em></td>
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<td><em>Health Minister or Mayor of Stavanger TBC</em></td>
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### Saturday 8 October

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>08.00-19.00</td>
<td>Exhibition; Poster Display; Registration; Hospitality Desk</td>
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<tr>
<td>08.30-10.00</td>
<td><strong>Industry Symposium 3</strong></td>
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<tr>
<td>10.00-10.30</td>
<td>Tea/Coffee – Exhibition Area</td>
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<tr>
<td>10.30-12.00</td>
<td><strong>New Developments in Haemophilia Care</strong></td>
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<tr>
<td></td>
<td>Chair: Brian O'Mahony, EHC</td>
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<tr>
<td>10.30-11.00</td>
<td>Gene Therapy</td>
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<tr>
<td></td>
<td><em>Edward Tuddenham, University College London</em></td>
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<tr>
<td>11.00-11.30</td>
<td>Bi-specific antibody, antithrombin and other new developments</td>
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<td><em>Claude Negrier TBC</em></td>
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<tr>
<td>11.30-11.50</td>
<td>EAHAD perspective</td>
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<td><em>Cedric Hermans, Cliniques Universitaires Saint-Luc</em></td>
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<tr>
<td>11.50-12.00</td>
<td>Discussions</td>
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<tr>
<td>12.00-13.30</td>
<td><strong>Industry Symposium 4</strong></td>
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<tr>
<td>13.30-14.30</td>
<td>Buffet Lunch – Exhibition Area</td>
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<tr>
<td>14.30-16.00</td>
<td><strong>Inhibitors</strong></td>
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<td>Chair: Mike Makris, University of Sheffield</td>
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<tr>
<td>14.30-14.50</td>
<td>Inhibitors in previously untreated patients</td>
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<td></td>
<td><em>Flora Peyvandi, University of Milan</em></td>
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<tr>
<td>14.50-15.10</td>
<td>Mild Haemophilia</td>
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<td></td>
<td><em>Paul Giangrande, Oxford University</em></td>
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<tr>
<td>15.10-15.30</td>
<td>Acquired haemophilia</td>
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<td><em>Lazlo Nemes, Hungarian National Haemophilia Centre</em></td>
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<tr>
<td>15.30-16.00</td>
<td>Panel discussion</td>
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<td><em>Frits Rosendaal, Flora Peyvandi, Paul Giangrande, Lazlo Nemes</em></td>
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<tr>
<td>16.00-16.30</td>
<td>Tea/Coffee &amp; Poster Session – Exhibition Area</td>
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<tr>
<td>16.30-18.00</td>
<td><strong>Industry Symposium 5</strong></td>
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<tr>
<td>18.00-18.45</td>
<td>WFH Update</td>
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<td>19.00-19.30</td>
<td>Coach Departure for Dinner</td>
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<td>20.00-24.00</td>
<td>Conference Dinner</td>
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<td>08.00-13.00</td>
<td>Exhibition; Poster Display; Hospitality Desk</td>
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<td>08.30-10.00</td>
<td><strong>Clinical Debates</strong></td>
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<td>Chair: Radoslaw Kaczmerek, EHC Steering Committee</td>
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<tr>
<td>08.30-09.00</td>
<td>Gene therapy - a cure in 10 years, yes or no?</td>
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<td></td>
<td><em>Edward Tuddenham, University College London</em></td>
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<td><em>Mike Makris, University of Sheffield</em></td>
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<td>09.00-09.30</td>
<td>Extended half-life factors will be the standard of care, yes or no?</td>
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<td><em>Angelika Batorova, Slovakian National Haemophilia Centre</em></td>
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<td><em>Paul Giangrande, Oxford University</em></td>
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<td>09.30-10.00</td>
<td>Caesarian sections for carriers, yes or no?</td>
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<td><em>Rezan Khadir</em></td>
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<td><em>Elena Santagostino TBC</em></td>
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<td>10.00-10.30</td>
<td>Tea/Coffee – Exhibition Area</td>
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<td>10.30-12.00</td>
<td><strong>Industry Symposium 6</strong></td>
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<tr>
<td>12.00-13.30</td>
<td><strong>Patient-Reported Outcome Data</strong></td>
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<td>Chair: Declan Noone, EHC Data and Economics Committee</td>
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<tr>
<td>12.00-12.20</td>
<td>Haemophilia in Europe in 2016 - results of the EHC Survey</td>
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<td><em>Brian O’Mahony, EHC President</em></td>
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<td>12.20-12.40</td>
<td>Update on the CHESS project</td>
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<td><em>Jamie O’Hara, EHC Data and Economics Committee</em></td>
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<td>12.40-13.00</td>
<td>Update on the PROBE survey data</td>
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<td><em>Mark Skinner</em></td>
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<td>13.00-13.20</td>
<td>National registries</td>
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<td><em>Anneliese Hilger, European Medicines Agency</em></td>
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<td>13.20-13.30</td>
<td>Discussion</td>
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<td>13.30-13.45</td>
<td><strong>Closing Remarks</strong> (followed by light lunch – format TBC)</td>
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<td><em>Brian O’Mahony, President, EHC</em></td>
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