EHC Newsletter August 2015

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President and CEO Report

European Inhibitor Network

In our April newsletter we announced the launch of a new programme dedicated to people with haemophilia who have inhibitors (PWI), their family members and their caregivers. We are pleased to report that our work in this area has started. In June we hired Kristine Jansone to manage and lead this programme and invite you all to give her a warm welcome into our community (see article on pg 15). In June we also created an ad-hoc working group, led by our Medical Advisory Group Chair Prof Paul Giangrande to begin shaping the programme and in July-August we launched a series of needs assessments (for National Member Organisations (NMOs), clinicians and PWI) to better understand existing needs and how best to meet them. The results of these needs assessments will be presented during a parallel workshop at the EHC Conference in Belgrade, Serbia, which will help to inform and give final shape to the programme. The final programme and its components will be announced at the end of the year. This programme is made possible by an educational grant from Baxalta.

Survey and Round Table on Tenders and Procurement

In May the European Haemophilia Consortium’s (EHC) ‘survey of coagulation factor concentrates tender and procurement procedures in 38 European countries’ was published in the scientific journal *Haemophilia*. As we announced in our April newsletter, one of the main findings is that whenever clinicians and patient organisation representatives are formally involved in the purchasing process, the best product and best price are achieved. The EHC presented an overview of the survey results during its Round Table of Stakeholders on this topic, held in Brussels on June 15 in the International Press Centre (see pg 11). The event, hosted by Member of the European Parliament (MEP) Dr Paul Rübig (Austria) was attended by more than 50 participants and also featured a presentation from the European Commission on the new EU Public Procurement Directive.

Youth leadership

We held our second Youth Leadership workshop in Rome in July with 15 bright and vibrant young NMO participants from 12 countries (Azerbaijan, Bulgaria, Denmark, Hungary, Ireland, Italy, the Netherlands, Poland, Serbia, Slovakia, Turkey and the UK). They came eager to learn how to be effective volunteers in their local and national communities and inspired us with their passion. It was a short but intense weekend of great fun and hard work as they probed...
each other on sustainable volunteerism, effective communications, appropriate relations with pharmaceutical companies and strategic planning (see article on pg 8). Upholding a tradition we started last year, we invited three of them to participate in the upcoming live stage debate at our annual conference in Belgrade, Serbia. Titled “Status Quo vs One Direction – debating haemophilia care,” they will go up against key European clinicians and argue for clinical freedom and better treatment and care for women and PWI. We look forward to seeing these and all other participants again at our conference and other EHC events. Engaging with the potential future leaders of our community is one of the most enriching and important areas of our work. We are grateful for the educational grants from Baxalta and Novo Nordisk for the youth leadership workshop and Sobi for the conference debate, which make these initiatives possible.

European Reference Networks (ERNs)

In June the European Commission Expert Group on Rare Diseases (CERD) adopted a revised addendum (see our April 2015 newsletter), which sets the framework for 22 groupings of rare diseases and acknowledges the role of patient representatives as knowledge experts in ERN activities. At this meeting the CERD also provided an update on potential funding sources for the administration of ERNs, which would likely come from Member States, the EU 2015 Health Programme, Connecting European Facilities (CEF) Fund, Horizon 2020 and Structural Funds – but the exact source, composition and amount of the funding is still unknown. The first call for ERN applications is expected to come at the end of the year with an expected application deadline of March 2016, selection of proposals expected to take place in May 2016, assessment reports in September 2016 and approval and establishment of ERNs by October 2016. It is likely that there would be more than just one call for applications (given that not all rare disease grouping may be ready to apply by the end of this year), but it is unclear how many more calls there might be. In terms of frequency, it seems that the European Commission is considering potentially repeating calls for applications every one and half to three years.

International Society on Thrombosis and Haemostasis (ISTH)

The ISTH Congress in Toronto was notable for the progress reported on new developments in treatment of haemophilia. Longer-acting factor concentrates have now been on the market in the USA and Canada since last year while they await licencing in Europe. The scientific and standardisation committee sessions included discussion on real life experiences of clinicians with these products to date. There was a wide variation in viewpoints on several questions posed. In relation to which people with haemophilia (PWH) should be given prioritised access to these products, views ranged from children, those with poor venous access or poor compliance or adults who should be switching from on demand to prophylactic therapy. Several clinicians stated they would be reluctant to prescribe longer acting factor concentrates to previously untreated patients (PUPs) outside of clinical trials until more data is available. Updated information from the clinical trials for some of these products still under development were reported. Developments in gene therapy were also reported and novel approaches including platelet directed gene therapy and research to reduce the effect of the
immune response to vectors were discussed. For more information about this event, please consult our article on pg 38.

EURORDIS Members’ Meeting

We attended the Members’ Meeting of the European Rare Disease Organisation (EURORDIS) held in Madrid in May and actively participated in the programme, particularly in the sessions regarding Centres of Expertise and European Reference Networks ERNs, where we shared our experiences and best practices with the European Haemophilia Network (EUHANET) as the rare disease community begins to consider ERN application. Another noteworthy feature of the EURORDIS meeting was its launch of Rare Diseases International. Olivia Romero-Lux, EHC Steering Committee member, reports in more detail on pg 36.

EHC 2015 Annual Conference

We look forward to welcoming the European haemophilia community to Belgrade, Serbia, for our 2015 Annual Conference, which will take place in a few weeks’ time. This year will mark a special edition of our conference as we will also hold a pilot Leadership Conference ahead of this event. The Leadership Conference will be an NMO-only meeting that brings together senior and young leaders as well as NMO staff members to give them a platform to discuss common issues with their counterparts from other European countries. This year’s Leadership Conference will have three main themes: governance, funding and procurement systems. The pilot Leadership Conference was made possible thanks to an educational grant from Baxter, Pfizer and Sobi. As for our Annual Conference, this year’s programme will feature sessions on comprehensive care, gene therapy, women and bleeding disorders, complications in haemophilia, inhibitors and longer-acting products. The conference will also feature two interactive workshops on inhibitors and women in the bleeding disorders community where we hope to get some discussions going on actions to be taken at European level in both those areas (the full programme of our conference can be found on pg 48). Registration for the Leadership Conference is now closed, however, there is still time to register to the EHC Annual Conference via www.ehcconference.org. We look forward to seeing you there!

Updated advocacy video

As many of you will recall, last year we published an advocacy video to mark the launch of the haemophilia treatment recommendations that came out of the Wildbad Kreuth III initiative, also known as the EDQM Recommendations (European Directorate for the Quality of Medicines and Healthcare). As we announced in our last newsletter, these recommendations have been endorsed by the Ministers of the Council of Europe through Resolution CM/Res(2015)3. As a result, we updated our advocacy video accordingly and also used the occasion to offer our community a longer video (similar in length to the original) and a shorter one (approximately two minutes) to better serve the advocacy needs of different NMOs. You can find both videos on our YouTube channel (www.youtube.com/ehctvchannel) and our website. We invite you to watch and circulate them widely.
Comprehensive Care in Haemophilia: Psychosocial Support

Petra Bučková* interviewed by Laura Savini**

To continue our series on comprehensive care in haemophilia we talked to Petra Bučková, psychologist at the Brno University Hospital in the Czech Republic, where she tells us more about her experience of caring for people with bleeding disorders.

According to the guidebook on psychosocial support for haemophilia developed by World Federation of Haemophilia (WFH), psychosocial support is needed for individuals affected by a bleeding disorder and their families to gain a personal understanding of the condition and to develop strategies for coping with physical, mental, emotional and social challenges that may come with living with a bleeding disorder. Unfortunately, it seems that professional psychosocial support is not always available in centres treating people with haemophilia and that in this situation psychological support will be most likely be provided by nurses and other healthcare professionals.

This is, however, not the case at Brno University Hospital, which is a European Haemophilia Comprehensive Care Centre, certified by the European Haemophilia Network (EUHANET). This means that people with bleeding disorders treated there can access professional psychological support, which will be mostly provided by Petra.

When I first asked Petra about her work with people with bleeding disorders, she stressed that it is quite similar to that performed with patients suffering from other chronic conditions. She also noted that although the primary goal of the haemophilia centre is to look after the patient’s physical wellbeing, it is also important to take care of their emotional wellbeing. This is where the psychologist’s work starts.

The type of issues faced by people living with a bleeding disorder are many and diverse and can range from issues that can be addressed with short-term psychotherapy, such as, for example, improving family communication or addressing a child’s unwillingness to see a doctor. Short-term support may also be given to people who are hospitalised for several weeks or months and who are mentally exhausted from the strain of medical treatment and being away from home for so long. With regard to longer-term therapy, this will generally address issues related to deeper and underlying problems. For this Petra notes that sometimes the problem may have arisen irrespectively of whether the individual had a bleeding disorder or not. Generally speaking these issues relate to the individual’s ability to
develop coping mechanisms to deal with their disorder. Finally, psychologists also perform *crisis interventions* when an acute problem or stressful situation presents itself. Above all, Petra stresses the importance of a close and supportive family unit, which, in her opinion, can help to overcome most problems. This is why *family therapy* should be provided when needed.

Petra follows patients at different stages in their lives, each one giving rise to different issues. For instance, she will work with young parents to address their anxiety of having a child suffering from a bleeding disorder; she will then work with children and adolescents who will need help to accept living with a chronic condition. She will also ensure that young adults learn to live responsibly and to care for themselves while ensuring that the parents understand that they can no longer control their children and need to let them live independently. Petra notes that many young adults stop visiting the centre as frequently as they should and sometimes tend to neglect their own welfare as a way to challenge their condition. In some rarer cases, some of her patients will develop hostile behaviours as a means of coping with the frustrations that come from living with a chronic medical condition.

Although any patient in the Czech Republic can access psychosocial services, as these are covered by the health insurance system, Petra’s patients are often referred to her by a haematologist (she works closely with Dr Jan Blatný who heads the Brno Paediatric Haemophilia Centre and Prof Penka, who is leading the service for adults). Despite this, Petra deplores the lack of systematic psychosocial follow-up of patients living with a chronic condition. From her professional experience, she sees many patients with complex problems developing later in life that could have been prevented by seeing a trained psychologist at a younger age.

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*Psychosocial support is needed for individuals affected by a bleeding disorder and their families to gain a personal understanding of the condition and to develop strategies for coping with physical, mental, emotional and social challenges that may come with living with a bleeding disorder.*

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As with other aspects of comprehensive care for people with bleeding disorders, Petra notes that there are great generational differences. For instance, with the advent of prophylaxis and higher trough levels, younger generations of haemophilia patients live an almost normal life. Young people with haemophilia still need to be careful in certain aspects of their life, for example, when practicing sports, but otherwise their life is much like that of any of their peers who do not have a bleeding disorder. Petra stressed that healthcare professionals in general are trying to ensure that younger generations stay active and realise that they can live a normal and productive life. This is due to the fact that unlike their younger counterparts, older generations of people with haemophilia still have to deal with pain and limited mobility brought on by more frequent bleeds. These individuals, she notes, are often in pain (both physically and emotionally) and are less integrated in society due to the disability and the fact that they cannot take part in regular activities. This leads to feelings of low self-esteem, isolation and helplessness. She stresses that it is important that younger generations realise that with good treatment they are not as limited as older generations and they can live life to the fullest.
Besides the support of a psychologist, patients and their families can also meet with doctors and nurses to discuss everyday problems, including psychological issues. For instance, every year the Brno University Hospital organises an open day where patients and their families can have private meetings with doctors to discuss current issues and receive guidance on disease management. Doctors and nurses also take part in summer camps organised by the Czech National Member Organisation (NMO) and according to Petra, these events are very important for children to learn how to behave safely when practicing sports and other common activities. During this time, physicians, physiotherapists and nurses also give short lectures on disease management, have informal interactions with patients and build long-standing relationships. So, even though psychosocial support is not systematic, patients have many opportunities to get additional support if needed. Finally, Petra stresses the importance of good collaboration amongst colleagues in the haemophilia comprehensive care team that allows her and her colleagues to talk about different medical aspects of common patients and quickly identify whether a particular patient needs additional healthcare services.

In terms of professional training, Petra notes that there is currently no specialisation for psychologists working with people with bleeding disorders as there is currently no professional society or network at the European level for psychologists working with people with haemophilia. She hopes that this can be developed in the coming years as she thinks it would be useful for sharing experiences and learning from peers across different countries.

Petra concluded the interview by saying that she finds working with people with bleeding disorders particularly interesting because, in her opinion, she can clearly see a correlation between stressful emotions and spontaneous bleeds. Through her work she feels that she can positively contribute not only towards an individual’s emotional wellbeing, but also see this translate directly into physical improvement.

*Petra Bučková is a psychologist working in the Brno University Hospital, Department of Clinical Psychology assigned to the Department of Internal Medicine, Haematology and Oncology.

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

Young volunteers meet in Rome for the second edition of the EHC Youth Workshop

By Laurence Woollard, Youth Ambassador, the Haemophilia Society UK

Earlier in July the European Haemophilia Consortium (EHC) organised its second Youth Leadership Workshop. Laurence Woollard, a young volunteer from the United Kingdom (UK), reports on the experience.

This was my first time abroad as a voluntary representative of the Haemophilia Society UK and what better place to visit than the “Eternal City” of Rome.

Having navigated the metro using the little Italian I know (and with a cockney\(^1\) twang), I arrived at the aptly named Leonardo da Vinci hotel, the location of our two and a half day Youth Leadership Workshop. By chance, I stumbled into a lovely girl called Karola from Hungary, who I soon realised was one of fourteen other representatives from different EHC National

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\(^1\) English dialect from London.
Member Organisations (NMOs) attending the event. With a few hours to spare, we braved the sizzling Italian heat to visit the city’s landmarks, such as the Colosseum, sharing each other’s experiences of growing up with a rare bleeding disorder along the way. This gave me much entusiasmo for things to come!

The evening’s welcome reception was a great opportunity to meet the other attendees, delegates and EHC staff. To break the ice, we had to draw a name from a bag, find the person in the room and ask a set of questions; we would then feedback the answers to the rest of the group the next morning. Coincidentally, I picked Karola and as we had already spent the day together, I had the chance to mingle with everyone. Although there were some language barriers, with delegates speaking a variety of languages from Polish to Azeri, I was overwhelmed by the sense of community and how our rare condition was easily translatable between us.

On the first full day of the workshop, an insightful introduction about the EHC was preceded by a short exercise that involved a simple ball of yarn. We formed a circle and were instructed to toss the wool to someone who shared the same motives for volunteering, taking hold of the thread each time. To our surprise, an intricate web began to form, holding us all together and causing us to move around the room as one. I really grasped the importance of building a network and understood that by cooperating together we can achieve our common objectives.

The next session focused on engaging and retaining volunteers. As volunteers ourselves, we recognised the value of our service and how volunteers’ support is crucial to the success of an NMO. However, attracting people to volunteer can be quite difficult. In teams, we explored different case studies, including ways to identify and recruit people to form a new youth group. One idea was to
hold an annual volunteer recruitment event, perhaps tying it in with an existing social event in the NMO’s calendar.

After working up an appetite, we were treated to the delicious local cuisine. Over the eight courses (not unusual I’m told), we shared personal stories of bleeding episodes and drew comparisons between our respective NMOs. Regardless of our backgrounds, it was apparent we were all present because we are passionate about improving accessibility to the support provided by our societies in our respective countries.

Before we knew it, it was our last day and the sessions centred on internal communication within an NMO. We were given an insight into the Irish Haemophilia Society and how they promote inclusiveness, participation, development of new ideas and ensuring everyone has a voice, an appropriate approach for the weekend seeing the Workshop’s motto being Speak Up, Stand Out & Join the Conversation. It gave us confidence that with positivity, patience and persistence, we can support our own organisation’s growth whilst remembering success is the sum of small steps.

Perhaps the most challenging activity of the workshop followed when, through role play, we simulated the positions taken on by more experienced members of an NMO. For instance, being the board member in charge of relations with pharmaceutical companies. I felt really out of my comfort zone but it gave me a greater awareness of leadership and the processes behind decision making. We concluded by devising our own action plans with my ultimate goal being to organise and run a summer camp with my NMO in 2016.

I relished the opportunity to represent my NMO and the workshop exceeded my expectations with the positivity and inspiration that I came away with. When I considered volunteering, I never envisaged the possibility of forming lifelong friendships with other people with haemophilia of various nationalities and learning the skills to become an effective youth leader. Since the workshop, I have been looking at ways to run a teen weekend with my NMO in 2016.
EHC holds Round Table on the results of its 2014 survey on the organisation of tenders and procurement for coagulation factor concentrates in 38 countries

By Laura Savini, EHC Communication and Public Policy Officer

Following the successful World Haemophilia Day (WHD) event in Dublin, it was with much excitement that the European Haemophilia Consortium (EHC) held a Round Table on 15 June on the results of the 2014 EHC survey on how coagulation factors concentrates are purchased and the role of patients’ organisations in the purchasing process in 38 European countries.

The results of this survey were only preliminarily presented during the WHD event and this was because we were still waiting for the survey to be accepted for publication in the scientific journal *Haemophilia*. The article has since been published and can now be consulted online [here](#).

The topic of the event was even timelier as there is a growing trend in Europe of countries looking into making joint purchases of medicinal products to reduce costs, which would most likely imply the use of tenders or some centralised purchasing system. This raises questions of how to divide the purchased products amongst different countries and healthcare providers, how to monitor product use and how to evaluate the efficacy of the product.

Furthermore, Member States are in the process of transposing the newly revised EU Public Procurement Directive². As we learned from one of our speakers, Mr Jaroslav Kracun, from the European Commission, the Directive may be applied not only to public entities but also to private ones that fulfil a public service role (for example, private health insurance funds), although this will depend on the interpretation of the legislation in each country. Finally, the movement towards patient empowerment and the inclusion of patients’ voices in healthcare decision-making is an ever growing topic on the EU scene as discussed by Aislin Ryan in her article on the launch of the “Patient Empowerment Campaign” by the European Patients’ Forum (EPF – see pg 28).

It was with this backdrop that the EHC welcomed over 50 participants to its Round Table held at the Residence Palace, Brussels’ International Press Centre; even though the room layout did not provide participants the opportunity to seat around the proverbial round table.

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² Directive 2014/24/EU
For a detailed account of the event, you can read the full EHC Round Table report (or for the less courageous, the executive report should suffice), which will be shortly available on our website. Here, however, we give you a few salient points discussed during the event.

The original survey split respondent countries in two categories: those with a centralised tender and those with alternative purchase processes. An additional split came with countries involving patients and physicians in the purchasing process versus countries that do not involve patients or physicians. Each side of the story was presented during the event by physicians and patients speaking about their involvement or non-involvement in the purchasing process and two physicians describing their experience working in an environment with and without a national tender.

You will probably not be surprised that countries with a tender and countries that include patients and physicians in their purchase process came out more positively than countries that do not have a national tender in place.

**How so?**

Well, it turns out that, contrary to popular belief, countries that involve patients and physicians in the national tender (and that incidentally have a specific regulatory framework in place) purchase the same product for less. Of course one needs to account also for other factors that may influence this, such as the economic situation in a given country, its pricing and reimbursement system and its ability to negotiate with manufacturers of medicinal products. However, the survey conducted by the EHC in 2014 showed that for a given product prices can differ as much as 400 per cent between two countries in the European region. The question that speakers and participants tried to address throughout the event was, why do these elements matter?

**Here are a few ideas**

**Tenders versus alternative processes**

As we learned from various presentations on the day, organising a national tender for haemophilia products implies much more than the organisation of the tender *per se*, which is already a major enterprise, according to Prof Paul Giangrande (Oxford University) who spoke about his experience as a healthcare practitioner going from no tender to a centralised tender in the United Kingdom.

Countries with a national tender organise their haemophilia care in a more central and comprehensive manner. The country will be more likely to have, for example, a centralised
registry and therefore have more data on the exact number of patients, their treatment regimens, and their general state of health, all of which provide governments with essential information to plan for product supply in the years ahead. Additionally, a single tender generally implies that the country has a single haemophilia strategy and budget, which can be used to better allocate resources. This way of working will also give treatment centres more autonomy from the hospital where they are hosted, as they will generally receive funding from a centralised budget. Moreover, this situation provides a greater safety net for the haemophilia centre as it protects the centre from any budget cuts linked to hospital performance and organisation. This centralised system will also most likely result in having specialised official treatment centres offering targeted services in a few key locations. Prof Cédric Hermans (Catholic University of Louvain) explained that in Belgium, as there is no formal national certification for haemophilia treaters and any healthcare practitioner can prescribe treatment for haemophilia and other bleeding disorders. This situation can result in less-than-optimal treatment for patients and potential product misuse and waste. Therefore it seems that a national tender is only one necessary element in providing a comprehensive and centralised organisation of haemophilia care services.

In alternative processes, the lack of coordination and central planning can result in huge amounts of money spent but not allocated in the most efficient ways. This was most incredibly demonstrated again by Prof Hermans who explained that although Belgium benefits from an estimated €10 million haemophilia budget, most of it is spent on medicines leaving haemophilia centres and services largely underfunded.

Patient and physician involvement versus non-involvement

As for patient and physician involvement, much progress remains to be made with only 14 out of 38 countries involving patient organisations in their tender process and 26 out of 38 countries involving clinicians. It is believed that the reason the involvement of these two groups lowers the final price (and increases access to treatment for patients) is that each of these groups brings a specific expertise to the topic. Generally speaking, officials in charge of tenders do not have any particular disease-related expertise but will primarily be specialised in the practical and legal aspects of tender organisation. Therefore, patients and physicians are an added value as they provide information on the condition, how it is treated and managed and what is needed for optimal treatment. Furthermore, they will also provide expertise on what processes companies need to put in place to ensure the safety and quality of medicines.

On this note, Mr Miguel Crato, President of the Portuguese National Member Organisation (NMO) noted that one of the main objectives of the NMO is to ensure that safety and quality criteria are taken into account when organising tenders so that officials do not solely rely on financial criteria, such as price.
This is important as it cultivates confidence and trust in the healthcare system among its users. The contrary can result in mistrust as noted by Dr Edward Laane (North Estonia Medical Centre) who recounted how corruption scandals in the procurement of haemophilia treatment in his country, Estonia, led to low levels of mistrust and much discontentment. The fact that neither patients nor physicians were involved in the tender process at the time of these scandals did not improve public opinion either.

**On the legislation itself**

During the Round Table, we also learned that the new EU Public Procurement Directive will indeed allow public entities to purchase solely based on cost. However, public entities can also decide to add qualitative criteria, which the EHC sincerely hopes they will do, when purchasing medicinal products. This shows that there continues to be a need for advocacy. On that note, Mr Brian O’Mahony (EHC President) stressed the importance of collaboration between patients and physicians towards achieving common goals.

**What about longer-acting products?**

If you are a regular reader of the EHC’s news (and a haemophilia patients yourself), you are probably already aware that longer-acting products are set to come to Europe in the next few years. If you are not, I would suggest you get in touch with your NMO and register for our workshop on New Technologies in Haemophilia Treatment taking place this November!

As this is an important and exciting topic for the haemophilia community, the event could not have ended without taking into consideration the impact that these new products will have on the organisation of tenders. Prof Flora Peyvandi (University of Milan) walked the audience through some of the key questions tied to the introduction of these products in Europe including: *Which products should be used for which situations? Should clinicians and patients favour higher trough levels or less infusions? Can the use of longer-acting products reduce expenditure in haemophilia care?* Her conclusion is that a European conversation on the subject must be started amongst both physicians and patients as there are no set answers.
An EU perspective

The event was concluded by Dr Paul Rübig, Member of the European Parliament, who kindly hosted the event (which could not take place in the European Parliament itself due to security restrictions). As the chair of the European Parliament Science and Technology Options Assessment, Dr Rübig has a keen interest on all matters related to science and technology with a particular interest in health. Dr Rübig noted the importance played by European companies (particularly small and medium sized ones) in bringing Europe to the forefront of innovation and giving European patients better access to new technologies. Dr Rübig is also the member of a small informal group of parliamentarians called Friends of Haemophilia and as such is always interested in exploring what can be done in the Parliament to improve access to treatment.

What next?

The EHC will continue to promote the results of this survey; however we also hope that our members will now take these messages home and use them at national level to advocate for better inclusion and representation in the tender or any other purchasing processes.

The next EHC Round Table will take place on Monday 30 November and will be on Mild and Moderate Haemophilia. For more information, please contact laura.savini@ehc.eu

Meet Kristine Jansone, EHC’s new European Inhibitor Network Programme Officer

Kristine Jansone* interviewed by Laura Savini**

At the end of June 2015, the European Haemophilia Consortium (EHC) team welcomed Kristine Jansone as programme officer to coordinate the EHC’s newly launched European Inhibitor Network. Laura Savini interviewed Kristine to learn more about her background and current work.

Kristine comes from Latvia, but has been living in Brussels, Belgium for the past six years. She has an academic background in Theology and the Science of Religion as well as Pedagogy. Before joining the EHC, Kristine worked as the General Secretary for the Ecumenical Youth Council in Europe (EYCE), a volunteer-led European youth organisation. When
her mandate ended, she looked for new challenges, both for professional and personal
development, and found herself on the doorsteps of the EHC.

Although she does not have any direct experience in public health, Kristine has been involved
in a variety of volunteering and professional activities for many years with a focus on social
inclusion and equality. She is passionate about human rights and solidarity, which is why she
was immediately attracted to the work of the EHC. In her opinion, advocating for better access
to treatment helps to ensure that people affected by haemophilia have access to basic human
rights. Kristine was also inspired by the idea of a patients’ network based on cooperation
towards achieving patient empowerment, education and training.

In her position, Kristine will be coordinating the European Inhibitor Network (EIN). The
programme, launched this year, will focus on bringing together and offering support to the
most isolated group within the EHC community – people with inhibitors. The EIN will seek to
identify the needs of people who have inhibitors, as well as their families and caregivers, and
develop activities and resources to help address these needs.

“It will be a great challenge in many ways, starting from my lack of familiarity with the
haemophilia and rare bleeding disorders community. There will also be the challenge of finding
out which is the best way to reach out to people who can be very isolated, both emotionally
and physically, and to build on the work of National Member Organisations to ensure that the
voice of people with haemophilia and inhibitors is heard at the European level.”

Kristine has already started to tackle her first challenge in her initial weeks at the EHC where
she learned a lot about haemophilia and deconstructed many myths and stereotypes about
the condition. The second challenge will be more difficult, but also more rewarding and
inspiring, as she puts it: “I truly believe in the power of people when they stand in true solidarity
and cooperation.”

For Kristine, working with the EHC is an excellent opportunity to match the knowledge and
skills gained in the international environment with the challenges of entering into a new and
unknown community and area of work.

Kristine is based in the EHC office in Brussels and can be reached at kristine.jansone@ehc.eu.

The EIN is made possible by an educational grant from Baxalta.
NMO News

NMO Profile: Azerbaijan from 1999 to today

By Gulnara Huseynova, President, Hemofiliyalı Xastaların Respublika Assosiasiyası (the Azeri NMO)

For this edition of the EHC Newsletter we asked Gulnara Huysenova, President of the Azeri NMO, to talk to us about her experience of sharing her life with people with haemophilia (her brother and her son) in a country with poor access to treatment. She also gives us an overview of the origins of the association and its current activities.

Origins of the association

The day my son was born was such a glorious day for me and my husband. I was 22 at the time. It was a long-awaited dream. We called him Ayaz, which means ‘breeze’ in Azeri.

But two days later, the thing that I feared most in life for my child became evident. Ayaz developed a large haematoma on his head. Despite the doctors' efforts, his condition grew worse and worse until they weren't sure that he would even survive. I cried night and day, praying that this was all a bad dream that would suddenly vanish.

I did not want to accept that Ayaz had haemophilia. I would have preferred that he had been born a cripple rather than have this dreadful disease. I did not want to believe it. And then the doctors added salt to our wounds by telling us that if the baby managed to survive, he would be mentally handicapped.

My mother’s reaction was even worse than mine. She came to the hospital and when she realized the situation, she confided in me: “You know how much I’ve suffered with your brother. Don’t suffer like I did. Don’t bring the baby home! Leave him at the hospital. Even if they manage to save his life, don’t bring him home.”

But I could not do that. He was my baby, no matter what! I had conceived, carried and bore him. I could not abandon him. But my mother had good reasons for her advice. My younger brother Ali had been born with haemophilia in 1973. I was eight or nine years old at the time. Nine months later, the nightmare began and all normality in our household ended. Bruises began appearing all over his body. At first, we thought it was normal because he had very tender skin.

But then he caught a cold and my mom tried to treat him with a few nose drops. The sensation, of course, was uncomfortable, and my brother started rubbing his nose and that is when it started to bleed and bleed and bleed. We couldn’t stop the bleeding. Fifteen days passed. Poor child! He had lost so much blood and had gone through so much trauma.

Everyone panicked. We took him to a number of doctors, but no one could diagnose him correctly. Finally, we took him to the ‘Mother and Child Care Hospital’ in Baku, the capital of Azerbaijan, where they told us that Ali had haemophilia.

Haemophilia? What was that? In those days, even in America, they were just beginning to understand how to use factor VIII to make the blood clot. In the Union of Soviet Socialist Republics (USSR), there was no such thing as a clotting factor and so they transfused my brother with blood and plasma. But by that time, he had lost so much blood. They told my
mother how serious his condition was and that we had to be very careful. With haemophilia, the slightest bump or fall could cause internal bleeding. Sometimes, the internal haemorrhaging can occur spontaneously for no apparent cause at all.

Unfortunately, the blood accumulates in the joints, particularly in the knees and ankles, which swell enormously. The pain is excruciating.

After that, my parents were not able to live a normal life. To tell you the truth, I can't remember ever spending a normal holiday together with our family. My brother was always in the hospital. Even though my mother had graduated with excellent grades from the Institute of Economy, she could not pursue a career. Instead, she stayed at home to take care of her son.

They were forever in and out of hospitals both in Baku and Moscow. My father had received the first Master's Degree and first Doctorate in Law in Azerbaijan, but his career, too, was affected. When my mother was with our brother, Dad took care of us four girls: Tarana, Matanat, Sevinj and me.

When Mom herself got ill, my older sister would carry my little brother to school in her arms. He was seven and she was 15. His leg had fully atrophied; he walked with such difficulty and refused a wheelchair. Even later at twelve when he was offered a cane in Moscow, he would have nothing to do with it.

As my brother grew up, naturally he wanted to play and be as active as any normal child, but he always ended up with painful bruises every time. How can you tell an active child: "Don't run! Don't jump! Don't ride a bicycle! Don't play soccer! Don't climb trees!" That's like saying: "Don't be a boy!" When Ali was six, he fell and injured his knee while playing in the street with friends. The doctors here in Azerbaijan said that he should lie down and elevate his leg. Everyone from the USSR went to Moscow for treatment but even there, they knew so little about treating this genetic bleeding disorder. In the USSR, all they could do was transfuse plasma and cryoprecipitate, the substance in blood that facilitates clotting.

Ali's leg became immensely swollen and bent at the knee, which interfered with normal walking. Doctors thought they could straighten it out by infusing cryoprecipitate. But they didn't succeed and, today, he suffers from one leg being seven centimetres shorter than the other. He tries to conceal the situation with a shoe that has a thicker sole.

The Next Generation

Haemophilia does not manifest itself in women but they may be carriers, meaning they have a 50 per cent chance of transmitting the disease to their sons. When I got married, I suspected that I might be a carrier but was never checked. All of my sisters had healthy boys.

At first my son Ayaz was quite active and healthy-looking. I found him so cute and funny that whenever I looked at him, I could nearly forget his ravaging disease. My sisters warned me not to let him run or jump, but I would not listen. If anyone made the slightest mention of his illness, I became upset.

But serious problems began when he turned three. He started bleeding internally in his joints at the slightest injury or bruise. The pain was indescribably intense. I cannot imagine grownups being able to handle it better than he did.
During the Soviet period, the workforce was obliged to give blood. Haemophiliacs like my brother were provided with plasma and cryoprecipitate. There was no such thing as factor VIII that you could administer yourself when the child screamed in pain in the middle of the night.

But when the Soviet Union collapsed, we lost the infrastructure in our haematology centres. No longer were blood donations mandatory. Thus, if your child needed blood, you had to go and find someone personally who would be willing to go to the clinic and transfuse blood directly to the patient. Hopefully, the donor’s blood was disease-free and was not affected by blood-borne diseases such as syphilis, hepatitis or HIV. There were no reagents to test for such diseases. One could only hope for the best.

Additionally, there was no secure way to store blood. You had to transfuse it right away from the donor directly into the patient. Often there were not even blood bags, so the blood would be collected in glass jars. Furthermore, there was no dependable refrigeration or back-up generators when the electricity went off, which it did so often.

It was especially difficult for anyone in need of blood between the years of 1992 and 1996. What can you do when you see your own child suffering in front of your eyes, and you are helpless to alleviate the child’s pain especially when you feel guilty as a mother for being the genetic link that gave him this disease in the first place? Sometimes you feel it is impossible for your heart to break any more than it already has.

The situation got so desperate with Ayaz that all I could think of was to try to find a way to take him out of the country to a place where he could receive treatment and live a normal life. For two years I made the rounds, knocking on every door of every organization and company I could think of, but to no avail. Nobody wanted to sponsor us.

But then a friend advised me to stop thinking about going abroad and focus my energy on setting up an organisation inside Azerbaijan, which could serve to help my child as well as others.

I had no options. There was no way out. I had to do something from within the country. That is when I started organising parents of people with haemophilia. In May 1997, we created the ‘Azerbaijan Republic Association of Haemophiliacs.’ Today, almost two decades later, over 1,400 people with haemophilia and von Willebrand Disease are registered from all over Azerbaijan. In 1999, we became a member of the EHC and in 2000, we became a member of the World Federation of Hemophilia (WFH).

Thanks to the attention and care of the head of state, President Ilham Aliyev, and First Lady Mehriban Aliyeva, the Ministry of Health of the Republic of Azerbaijan periodically take measures to solve the problems of patients with haemophilia.

The Ministry of Health has successfully implemented and enforced decrees and decisions of the head of state to improve the quality of life of patients with haemophilia, ensuring that the government budget would cover the purchase of coagulation factor concentrates. Moreover, the National Haemophilia Centre is now equipped with modern technologies for the diagnosis of bleeding disorders. Additionally, medical staff is trained to deal with various surgical procedures relevant for people with haemophilia.

The most recent haemophilia state programme (2011-2015) has implemented a series of actions; the most important were the provision of factor concentrates and the establishment of a national register for people with bleeding disorders to collect data on the distribution and use of coagulation factor concentrates and to evaluate the effectiveness of health care.
Thanks to these reforms, we have now been able to achieve 1.4 international units (IU) per capita of FVIII concentrates and to improve quality of life for people with haemophilia, many of who nowadays do not differ from healthy people.

As for Ayaz, I am happy to report that he is now a healthy 26-year-old working as a dentist. He is also active with the Society and recently took part in the EHC Youth Leadership Workshop (see article pg. 8).

About the Azeri NMO

As for the Azeri haemophilia association, our current objectives are to promote the quality and safety of haemophilia care as well as social and medical rehabilitation in Azerbaijan. In this regard, we actively collaborate with the government to ensure that safe and effective coagulation factors concentrates are purchased. Our organisation also aims to improve the level of social protection for people with bleeding disorders. In this regard we try to provide our members with legal advice on their rights and what they are entitled to. Like other haemophilia organisations, our objective is also to bring together people affected by the same disorder and we do so by developing social programs such as health-oriented camps. Finally, we promote safe and non-remunerated blood donation.

Our organisation is managed by a board of seven people, including myself. We also have a small office composed of an administrative assistant, a translator and an accountant.

Between 2001 and 2004, we took part in a WFH Twinning Programme with the Turkish Haemophilia Society.

Between 2004 and 2008, we completed the WFH Global Alliance for Progress (GAP) project, which allowed our medical professionals to gain additional medical training in the area of haemophilia and other bleeding disorders. Furthermore, our country was able to purchase laboratory and medical equipment and we received 600,000 IU of coagulation factor concentrates from the WFH Humanitarian Aid programme. During this project, representatives from our Ministry of Health attended the WFH Congress, workshops and seminars abroad on haemophilia and we even signed a Memorandum of Understanding. Furthermore, we organised several conferences and seminars in Azerbaijan to educate and raise awareness about haemophilia.

More recently, we started a two-year project in 2013 on ‘Decentralisation of care of haemophilia patients through education and capacity-building centres for the diagnosis of haemophilia.’ This project, funded by industry, aims to provide training and establish laboratories for the diagnosis of bleeding disorders in the cities of Baku, Ganja and Shirvan.
Hungarian National Member Organisation (NMO) elects new board

By Borbála Keszthelyi, board member of Magyar Hemofília Egyesület (the Hungarian NMO)

Earlier this year, the Hungarian NMO elected a new board. Borbála Keszthelyi, a newly elected member of the board, introduces herself and the other board members.

Dear Readers,

We would like to introduce the new board of the Hungarian NMO, composed of eight people: five working as volunteers in our board and three in our audit committee. Our NMO has more than 600 members and many volunteers are helping our Society in organising the summer camp, events and other gatherings.

The most important missions of our NMO are to effectively advocate on behalf of our members, inform our membership about the latest scientific and medical advances in the treatment of haemophilia and ensure access to treatment. Our aim is to create an effective and flexible communication channel where the board and the society members can communicate directly and share their experiences and thoughts. Our NMO has to build a service that is needed by the members, where each member can gain access to useful services in order to improve the quality of their life.

NEWLY ELECTED HUNGARIAN NMO BOARD

Zoltán Keszthelyi, President

Zoltán Keszthelyi graduated in Eötvös Loránd University (ELTE) as a programmer mathematician and software engineer. He works in Hungary at a multinational company as a software designer and adviser but he took international courses in informatics as well. He has severe haemophilia A. He is one of the co-founders of our NMO. He has a wonderful wife who he has been married to for 33 years and two beautiful daughters.

Attila Csaba, Vice-President

Attila Csaba graduated as a programmer mathematician and holds a degree in economics. He works as an economist, event organizer and accountant in Hungary. He has lot of experience in company management and leading a society. He has also been participating in other non-profit organisations.

Tímea Hadházy, Board Member

Tímea Hadházy is a mother of two sons, one with haemophilia A. She represents the parents of children with haemophilia and as such has strong ties with other parents in the community. Tímea is a psychologist and currently works as a human resources (HR) specialist in a multinational company.
Péter Nagy, Board Member
Péter Nagy is a student in mechanical engineering at Miskolc University and he has severe haemophilia A. Péter is a key member of the organising committee of the Rehabilitation Summer Camp for children. He participated in many international conferences and has good relationships with haemophiliacs from other countries. In his free time, Péter enjoys photography and bike riding.

Zoltán Molnár, President of the Audit Committee
Zoltán was the president of our NMO for many years. He currently works as a finance specialist and accountant in a Hungarian company. He has severe Haemophilia A.

László Tóth, Member of the Audit Committee
László is an economist and works as an accountant. He lives in the beautiful country town of Sopron. He has severe haemophilia A and he advocates for the needs of older people with haemophilia.

István Kovács, Member of the Audit Committee
István is an information technology specialist and he currently works in a Hungarian company. He has severe haemophilia A. He was a board member in our society previously and helped to organise our summer camp.

Borbála Keszthelyi, Volunteer
Borbála is responsible for the summer camp and communications with international organisations. She is an economist with marketing specialisation and currently works in a multinational company as an HR and marketing specialist.

Erzsébet Gömbös, specialist nurse
Erzsébet is a nurse in the Hungarian Orthopaedic Clinic and works as a mental health specialist. She has experience in the care of people with haemophilia with a particular focus on mobility issues. She is our liaison with healthcare organisations.
Meet Lino Hostettler, newly elected President of the Swiss National Member Organisation (NMO)

By Lino Hostettler, President, Schweizerische Hämophilie-Gesellschaft (the Swiss NMO)

Earlier this spring, the Swiss National Member Organisation (NMO) elected a new President, Mr Lino Hostettler. Lino is a regular participant of many EHC events (workshops, Annual Conference, etc.) and therefore you may already know him. For those who have not yet had the pleasure of meeting Lino, here are a few introductory words.

Dear Readers,

I would like to briefly introduce myself for those who do not yet know me. My name is Lino Hostettler and I have severe haemophilia A. I live in Zürich where I work in social insurances for a compensation fund. In my free time I play clarinet and I enjoy going to the opera and travelling.

I have been a board member of the Swiss Haemophilia Society since 2010 and as such I have continuously pursued the development of the Society. This has allowed me to develop my experience within the Society and to create many relationships in the haemophilia community well beyond Switzerland.

A recent survey in our membership shows that the need for a patient organisation is as great as ever and therefore I wish to succeed Mr Hans Meier as President to promote the development of our haemophilia community.

The EHC wishes Lino a successful mandate in his new position of President of the Swiss NMO.

World Haemophilia Day in Lithuania – First-ever basketball team of hemophiliacs

By Viktorija Jusinskaite, Lietuvos Hemofiljos Associjacija (the Lithuanian NMO)

The Lithuanian NMO has put together the first ever basketball team of PWH and had them challenge a professional team during the last WHD. Here we hear more about this experience.

The Lithuanian Haemophilia Society (LHS) works actively on different projects together with other countries. At the national level, the LHS helps patients to communicate with health care specialists to ensure better treatment of haemophilia. In the last eight years, the LHS has emerged as a strong community and achieved a lot, including access to prophylactic treatment with recombinant factor for all patients, joint replacement surgeries and qualified rehabilitation.
Thanks to prophylactic treatment, young Lithuanian haemophilia patients can be physically active and totally integrated in society. Physical activity is very important for every person but when we are talking about haemophilia patients, the benefits are even greater, especially when young patients have access to prophylaxis. However, it is often quite difficult to keep people affected by haemophilia physically active, due to a lack of motivation. Therefore, the LHS decided to create a basketball team of young haemophilia patients to encourage them to be more physically active and to build their self-esteem.

The basketball team was created in autumn 2014. Today there are 13 players with haemophilia from different cities in Lithuania. All team members practice twice a week to achieve better coordination, endurance and strengthen their joint muscles. Most team members had never played basketball before but their enthusiasm and motivation has given good results over the past six months.

The haemophiliac basketball team played its first competition on World Haemophilia Day. The challenger was a professional basketball team called Atletas. The next day, the LHS team attended the Lithuanian basketball league, where they had the honour to play with the best Lithuanian basketball club, Zalgiris. Despite losing both matches, the young team showed that great treatment can allow them to live active lives.
We challenge other countries to do the same!
Regional Haemophilia Symposium in Turkey

By the Türkiye Hemofili Derneği (the Turkish NMO)

On 8 June, the Turkish National Member Organisation (NMO) organised its 2nd Regional Haemophilia Symposium, which was attended by 116 delegates including patients and their families and physicians and nurses from Dicle University. The Turkish NMO reports on the event.

The one-day event was prepared by fifteen volunteers who travelled to Diyarbakir, a city located in the south-eastern part of Turkey. The symposium gave participants the opportunity not only to learn more about scientific advances in haemophilia treatment but also to seek medical advice from the healthcare specialists attending the event. In the morning, 41 patients were examined by Dr. Başak Koç and Dr. Fikret Bezgal. Afterwards, another examination followed at the Haemophilic Arthropathy Council, with the participation of Dr Bülent Zülfikar (Professor in Hematology), Dr Önder Kılıçoğlu (Professor in Orthopaedics) and Dr Gülsüm Ak (Professor in Dentistry).

After lunch the scientific programme started. Opening speeches were made by the General Secretary of the Turkish NMO, Mr Fikret Bezgal; the President of Dicle Haemophilia Association Council, Mr Selim Paksoy; President of Şanlıurfa Haemophilia Association Council, Mr Ali Yıldırım, the Representative of the Turkish NMO Şanlıurfa Province, Mr Mehmet Emin Akın, and the Provincial Health Director of Diyarbakir, Dr Mehmet Sait Avar.

The scientific programme was divided into two separate sessions, chaired by Prof Dr Bülent Zülfikar and Dr Orhan Ayyıldız (Professor in Haematology). The first session began with Prof Dr Orhan Ayyıldız’s presentation, which emphasized the importance of the Regional Haemophilia Symposium in educating patients and their relatives. Subsequently, Dr Murat Söker (Professor in Paediatric Haematology) talked about the importance of the multidisciplinary teamwork and haemophilia treatment centres in his presentation titled ‘Regional Differences and Problems of the People with Haemophilia’. Dr Başak Koç discussed the treatment of haemophilia, the importance of prophylaxis, the classification of factor levels and inhibitor reporting. Prof Dr Bülent Zülfikar stressed the importance of haemophilia treatment and prophylaxis, giving examples from across the world. Dr Zülfikar focused on the importance of prophylactic treatment, as well as diagnosis and diagnostic methods in prophylaxis. He shared with the participants the benefits and key role played by the HemofiLINE haemophilia registry system.
During the second session of the Symposium, Dr Ergül Berber (Associate Professor in Genetics) offered the latest information regarding gene therapy, receiving undivided attention from the participants. Dr Berber noted that gene therapy treatment for haemophilia B patients is still going on successfully. He also noted that current research is looking to extend this treatment to patients with haemophilia A. Prof Dr Gülsüm Ak discussed the importance of oral and dental health for patients and gave special attention to the correct procedures for maintaining impeccable dental hygiene.

Finally, Prof Dr Önder Kılıçoğlu gave a presentation on the treatment of orthopaedic problems in patients with haemophilia. He focused on the importance of multidisciplinary care and maintaining a healthy lifestyle. In addition, he also gave an overview of the most common orthopaedic procedures.

Following the presentations, delegates had the opportunity to fill in the gaps in a Question and Answer session.

The Regional Haemophilia Symposium concluded with a discussion on the challenges faced by haemophilia patients in Turkey. This gave the Turkish NMO a direction in which to steer the association’s activities in the future in order to address these challenges.

The board of directors of the Turkish NMO was delighted to present the representatives from Dicle University, Prof Dr Aysegül Jale SARAÇ (President of the University), Prof Dr Orhan AYYILDIZ and Prof Dr Murat SÖKER, with a commemorative plaque for their support and cooperation during the Symposium.
Feature Articles

EPF holds conferences on patient empowerment and cross-border healthcare

The EHC is a member of the European Patients’ Forum (EPF), an umbrella organisation comprised of patient organisations for chronic diseases from across Europe.

In May, the EPF launched a campaign on patient empowerment, which will be rolled-out this year and next to stress the importance of patient involvement in healthcare decision-making. Aislin Ryan, EAHAD-EHC Project Consultant, attended the launch conference and reports here about the event and campaign.

Meanwhile, over the past two years, the EPF has organised a series of mini-workshops in various Member States to provide training on the Cross-Border Healthcare Directive and how patients can benefit from it. In July, they organised a conference to report on the various events. Cynthia Bonsignore (former EPF Communications Officer) writes about the results of the conference and some of the comments by EHC members who attended the national workshops.

Patients prescribe empowerment

On 20-21 May in Brussels, the European Patients’ Forum (EPF) brought together patient organisation representatives, health professionals, national policymakers and academics to discuss the theme of patient empowerment. The conference titled ‘Empowered patients are an asset to society’ focused on defining patient empowerment, sharing best practices and discussing strategies for putting patient empowerment on the European Union (EU) policy agenda.

The conference began with an introduction to the concept of patient empowerment and why it is important. Patient representative Robert Johnstone reminded attendees that patient empowerment is a central concept in healthcare. He quoted the World Health Organisation’s (WHO) Alma Ata Declaration from 1978 that states, “The people have the right and the duty to participate individually and collectively in the planning and implementation of their health care.” However, he noted that even if this idea has been around for a long time, many patients do not experience it on a daily basis.

Prof Angela Coulter from the University of Oxford then spoke about the evidence-base for patient empowerment. She summarized a number of studies that have shown the positive impacts that result when patients are involved in decision-making about their own treatment, including better treatment adherence, fewer adverse events and less costly healthcare.

The next plenary session focused on case studies of how patient empowerment can work in practice. One talk that generated a lot of interest and discussion was that of Dr Camille Ratchke from the Danish Junior Doctors’ Association. She spoke about an initiative in Denmark called ‘Hello Healthcare,’ which encourages patients and their family members to ask questions as a way to promote better communication between doctors and patients. She pointed out that although it sometimes seems as though the doctor is the barrier to patient empowerment, in fact most doctors are very committed to putting the patient first. However,

they face constraints due to a healthcare system that is focused on productivity and efficacy,
which can mean doctors only have a very short period of time in which they can meet with each patient. She emphasized that patient empowerment must also be looked at from a health system perspective to see what changes can be made at that level.

After the morning plenaries, participants separated into three workshop groups each of which focused on a different component of patient empowerment: 1) health literacy and the informed patient; 2) the role of the patient–professional relationship; and 3) the role of self-management in chronic disease. The workshops were designed to be results-oriented with delegates working together to suggest ten guiding principles that should be included in a ‘Charter of Patient Empowerment’ and defining possible steps that could be taken to implement a patient empowerment strategy in Europe, referred to as a ‘Roadmap to Patient Empowerment.’ The workshop participants provided a variety of ideas on important topics that must be taken into consideration in such a charter. Throughout the next year, EPF will continue to work on both the Charter and the Roadmap to come up with specific actions to be taken by European policy-makers and others involved in the healthcare system to increase patient empowerment.

The conference was also the occasion for EPF to launch its new EU-wide campaign on patient empowerment. The slogan for the campaign is “Patients Prescribe E5 for Sustainable Health Systems.” Cynthia Bonsignore, former EPF Communications Officer, presented the campaign and explained that the slogan refers to the active role patients should have in their own healthcare. It is an opportunity for patients to share with health policy-makers and other stakeholders their perspectives on what empowerment means. E5 represents the five components of empowerment: education, expertise, equality, experience and engagement. EPF has developed a toolkit to help patient groups participate in the campaign at Member State level. This includes promoting the campaign using social media, writing letters to national representatives or organising local events to raise awareness about the campaign.

Materials from the conference and the campaign are available on EPF’s website at: www.eu-patient.eu

Cross-border healthcare

After a series of national and regional events held from 2013-2015, EPF organised a major European conference on cross-border healthcare in Brussels, Belgium, this past July. Patient leaders from across the EU and representatives from National Contact Points (NCPs) met to discuss the implementation of the Cross-Border Healthcare Directive4. It emerged from the discussions that patients, and their representative organisations, play a key role in ensuring the Directive works effectively.

“We find that a number of Member States appear to have done a good job of transposition. But there are others where we have a number of serious concerns” remarked Health Commissioner Vytenis Andriukaitis who also mentioned the need for a ‘fair’ transposition of

4 Directive 2011/24/EU
the Directive. Among the examples of barriers he provided, he mentioned very elaborate systems of prior authorisation, lower reimbursement tariffs or difficult administrative requirements.

**(In)Equity of access**

During the series of conferences it became evident that the implementation of the Directive by the Member States was still disparate.

Financial barriers are a major threat to equity of access: many patients are not able to afford cross-border healthcare due to the requirement for upfront payment of the treatment and having to cover other related costs, such as travel.

Member States and National Contact Points are asked to proactively work on solutions to alleviate the financial burden on patients, particularly those who are less well-off financially.

**Information, the keystone**

Information, or the lack of it, came to the forefront of the event discussions. The establishment of NCPs to provide information was patchy and patients reported being faced with ‘a labyrinth of confusing, sometimes insufficient and sometimes too-detailed information.’

“*Implementing the Directive requires all stakeholders to create a culture of transparency and cooperation where they disclose information specifically on how they operate; the availability and cost of care and its safety and quality. This will in turn help patients who are already vulnerable due to ill health and so may not have the capacity to battle bureaucracy to make use of their rights,*” said EPF Secretary General Nicola Bedlington.

EPF calls for EU-wide guidelines on how NCPs should provide information to patients in terms of harmonisation of forms, templates, information on safety and quality, etc. They need to work with patients to ensure that the information they provide meets their needs.

“*Our role is to ensure that patients have the right information they need to make informed decisions about cross-border healthcare: information BY and FOR the patient,*” said Sinisa Bosnjak, the Slovenian Contact Point during the event.

**Transparency, the changer**

The transparency requirements of the Directive are potentially a huge step towards patient empowerment, the theme of EPF’s major campaign for 2015. Patients will be able to compare standards between different countries and patient organisations can use this information to advocate for better quality healthcare, both at home and abroad.
However, the Directive does not contain a definition of quality or safety. This is an area where patient organisations can play a valuable role, including by participating in EU level discussions around a common understanding of quality of care.

“A space has emerged for patient organisations to inform patients of their rights and how to go about exercising them, but also to help improve the patient journey overall by working more closely with competent authorities and NCPs. Patient groups now have a clearer view of the intention behind the words of the Directive and are already taking action to even become unofficial contact points in France or Bulgaria for instance,” concluded Ms Bedlington.

What’s next?

In the autumn the European Commission will publish its implementation report. EPF will follow this very closely and take a position on what needs to be improved in consultation with our members and the network of patient leaders.

Feedback from EHC members on the national workshops

“The European Patients’ Forum day on cross-border healthcare legislation was really interesting. I knew nothing about people’s right to be treated in another EU country and to hear about how it works in practice was useful. However in the UK we have really high quality haemophilia and orthopaedic care, so the most common things people need are generally available here. However, there are some treatments that people have to wait a long time for, so this could be a useful initiative to speed up people’s care if their treatment is available here, but there is a long waiting list. It is certainly worth knowing about if the treatment you need is available in your country, but there is a very long wait and it is available at the same or lower cost elsewhere.” Liz Carroll, Chief Executive Officer, UK NMO

“The event showed clearly the need for better dissemination of policy rules within patient associations and the work carried out by National Contact Points (NCPs) in different countries as well as the EPF for a better clarification to their members. The event gave us good information on how to access cross-border healthcare and covered many interesting topics such as its cost and reimbursement for patients, the quality and safety of treatment, assistance for patients with special mobility needs and third-party tracking, the medical post intervention, different forms and medicines between the EU and possible side effects resulting from the intervention.” Carlos Mota, Board member, Portuguese NMO
Irish Haemophilia Society organises hepatitis C conference

By Declan Noone, Chair EHC Data and Economics Committee and staff member of the Irish Haemophilia Society

In June 2015, the Irish Haemophilia Society (IHS) organised a European Hepatitis C (HCV) Conference in Dublin to provide a forum for transferring knowledge among people living with both haemophilia and HCV. Declan Noone reports.

The conference was specifically tailored to countries where new HCV treatments are currently available. The two-day event also covered current levels of access to treatments and advocacy activities in participating countries. The conference was attended by both medical professionals, such as hepatologists and haematologists, and by patients’ representatives from 27 European countries. The IHS was proud to welcome a grand total of 65 delegates to this conference. The programme included an overview of haemophilia and HCV in Europe, a very detailed update on current and future treatment options, and an overview on access to treatment in several European countries.

Currently, we know that in 29 European countries (20 of which are in the European Union), there are an estimated 41,142 people living with haemophilia and, of these, 10,457 report having HCV. Numbers are unfortunately high in some countries. For example, in the United Kingdom (UK) we estimate that approximately 1,298 people with haemophilia are also infected with HCV. These numbers go up to 1,501 in Italy and even to 1,730 in France! However, these figures are still relatively small when compared to the total population of people infected by HCV in some European countries: 12,365 people in Ireland, 688,000 people in Spain and an astounding 1 million people in Italy.

Unfortunately, the availability of the new generation treatment for HCV varies across Europe, as do the prices being paid for these medications. Success rates with the new treatments are very high and side effects are much more limited compared to old generation treatments. This has made it possible to see good treatment outcomes, even in people with the most advanced liver diseases. Current treatments being marketed have a success rate of over 90 per cent.

At the conference, physicians spoke on clinical aspects of HCV and the importance of treating it. Prof Michael Makris from Sheffield University presented the latest figures from the
European Haemophilia Adverse Events Surveillance System (EUHASS) that showed that liver diseases are currently the biggest single cause of mortality for people with haemophilia in Western Europe. This demonstrates that it is absolutely imperative to clear the hepatitis C virus in people with haemophilia.

Dr Diarmuid Houlihan from St Vincent’s University Hospital in Dublin discussed the clinical progression of HCV and the complications of end stage liver disease, which develop during cirrhosis. This includes varices, ascites (i.e. fluid in the abdomen) and encephalopathy, all of which can cause a lot of discomfort for patients. In a number of cases patients with hepatitis C also develop hepatocellular carcinoma (HCC or liver cancer), which requires treatment either through surgery, resection or transplantation. Alternatively, other methods such as thermal ablation or chemotherapy can be used in the interim to slow the progression of the liver cancer and gain time before ultimately performing a liver transplant.

Besides preventing the development of severe liver diseases, another reason for treating patients with HCV early is to improve quality of life and to prolong life expectancy after they clear the virus. Speakers noted that clearing HCV adds at least an additional ten years to the person’s life expectancy.

Prof Geoffrey Dusheiko, from the Royal Free Hospital in London spoke about the new era of HCV eradication and the treatments available in 2015 and beyond. There are eight treatment options available under the guidelines developed by the European Society for the Study of the Liver (EASL), which are used to different degrees in different genotypes.

Prof Dusheiko gave an excellent overall summary of the current state-of-the-art treatment. In general, patients who have no cirrhosis or genotype 1b are achieving sustained virologic response (SVR) rates between 95-100 per cent. The next group are the cirrhotic patients who have Childs-Pugh score⁵ A categories, with genotype 1a or harder to treat 1b’s. These patients are achieving SVR rates in the 90-95 per cent range. The third cohort is those with cirrhosis

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⁵ The Child-Pugh score is used to assess the prognosis of chronic liver disease, mainly cirrhosis.
who are in the Childs-Pugh B and C categories. This group is achieving SVR rates of 85-90 per cent. Finally, the group with the lowest success rates, achieving less than 85 per cent, are those with genotype 3, those who are treatment experienced and those in Childs-Pugh C categories. However, with these new treatments and high success rates it should be entirely possible to envision the eradication of HCV in the haemophilia community.

In the Saturday afternoon session, the delegates had the opportunity to present on the current situation and the level of access to treatment in their own countries. Delegates from Canada, Denmark, France, Germany, Ireland, the Netherlands, Poland, Portugal, Scotland, Serbia, Switzerland and the United Kingdom all presented on the state of access to new HCV treatment. Some countries currently have no access to the new treatment and are unfortunately still using interferon-based treatments for all patients. However, most of the countries had some access to the newer treatments. Generally speaking, in almost all countries priority patients are those with cirrhosis (late stage or not), those with F3 fibrosis and accelerating factors such as co-infections with HIV or hepatitis B (HBV), as well as patients that went through liver transplant or have other medical problems.

Speakers noted that clearing HCV adds at least an additional ten years to the person’s life expectancy.

From the discussions, it emerged that the three countries with the most interesting and innovative treating protocols were Canada, Portugal and Scotland. In Canada, patients with haemophilia are entitled to treatment regardless of the stage of their liver damage, as treatment is provided under the HCV compensation scheme that was put in place for people with haemophilia. In Scotland, over the last five years there has been a policy of making the treatment of HCV a national health priority. This is paying off with some regions of Scotland literally running out of patients to treat under current criteria and needing to move on to those with little or no fibrosis. Although access to treatment is still difficult, it is becoming increasingly widespread and there is hope that HCV will be eradicated. The biggest surprise came from the Portuguese healthcare system, where over 1000 patients have been treated since the start of the year! This has been achieved thanks to a coordinated national system, where all patients treated are entered into a national registry to track the cure rate so that performance can be improved. This was made possible thanks to the pressure put on the government by the press and to agreements made with pharmaceutical companies where the price per patient is reduced over time. It is these sorts of nationally co-ordinated efforts with long-term plans and goals, similar to what we are seeing in Scotland that will make real progress towards HCV’s eradication.

Next participants were shown a preview of the latest report from the Irish Health Protection Surveillance Centre on HCV. This database looks at the progression of HCV in people in Ireland who were infected by blood products supplied by the state. This includes people who were infected through anti-D immunoglobulin, blood transfusions, kidney dialysis or treatment for haemophilia. The database demonstrates, as indeed did previous reports, that the clinical progression of HCV is more rapid and severe in people with haemophilia and in those infected by transfusions. Data shows that in these populations there is a higher proportion of clinical signs of serious liver disease, cirrhosis or liver cancer, all of which result from the clinical
Another significant factor linked to more rapid progression of liver disease is high alcohol intake, which increases the risk of serious liver diseases fivefold. On a positive note, the database also shows that the progression of liver disease can be greatly reduced by successful treatment for hepatitis C and by lowering or ceasing alcohol intake.

The final session of the conference focused on optimising support for members of haemophilia organisations. In many cases haemophilia organisations are working not only towards getting access to the new treatments but also towards socially and emotionally supporting people who are preparing for or undergoing treatment. This is of particular importance due to heavy side effects that can alienate patients and make them more vulnerable. Participants spoke from experience on the importance of developing a social network with whom to share the experience of going through treatment. The session also highlighted the important role that can be played by the patients’ organisation in mentally preparing patients for the treatment process. In addition, the importance of maintaining contact with members after they have achieved SVRs, especially if they still have cirrhosis, was discussed. Attention was also drawn to the need to provide support for patients who are currently waiting for transplants or those for whom even the new treatments have failed.

Overall, the weekend emphasised the importance of obtaining access to the new treatments and offered delegates an excellent opportunity to share experience, knowledge and ideas on how to advocate for the new treatments.

The Irish Haemophilia Society released on 28 August its latest edition of Positive News, a newsletter dedicated to hepatitis C and haemophilia. In this issue, readers can learn, among others, more about the 2015 Annual Conference of the European Association for the Study of the Liver (EASL) together with EASL’s latest guidelines to for treatment of hepatitis C. Positive news can be consulted online on the website of the Irish Haemophilia Society. For more information look at our publications section at the end of this newsletter.

Eurordis Membership Meeting 2015

By Olivia Romero-Lux, EHC Board Member

This past May, Olivia Romero-Lux attended the annual membership meeting of the European Organisation for Rare Diseases (EURORDIS). She gives an update on key topics discussed at the meeting.

From 28-30 May, the 2015 EURORDIS Annual Membership Meeting took place in Madrid. The event kicked off with the election of four new board members: Ms Simona Bellagambi from UNIAMO, the Italian Association for Rare Diseases, Ms Françoise Salama from AFM Telethon, Mr Nick Sureau from AKU UK, a patient organisation for alkaptonuria and Ms Avril Daly from Genetic and Rare Disorders. The event continued with the presentation and approval of the 2014 activity and financial reports, followed by strategic planning for 2015-2020. The 250 delegates taking part in the event then had the opportunity to participate in different workshops and plenary sessions.
Launch of Rare Diseases International

Alongside the Membership Meeting was the launch of a new international rare disease organisation. Rare Diseases International (RDI) is a global network, representing patients with rare disease. Over 20 national organisations have signed up so far, including the National Organisation for Rare Disorders (NORD), the Canadian Organisation for Rare Disorders (CORD), as well as organisations from Japan, India and China. Organisations from another 50 countries are expected to join. During the launch of RDI, the principles of a Joint Declaration were adopted by over 60 attendees. This new platform will help groups develop rare disease policies in their countries to adopt best practices and shall make the voice of patients heard worldwide. The main objectives of RDI are to 1) promote rare diseases as an international public health and research priority by raising public awareness and influencing policy-making; 2) represent its members and people living with rare diseases at large, in international institutions and fora; and 3) enhance the capacities of its members through information, exchange, networking, mutual support and potentially joint actions.

European Reference Networks

The plenary session of the meeting focused on European Reference Networks (ERNs). ERNs provide a clear governance structure for knowledge- and best practice-sharing and care coordination across the European Union. They are networks comprised of centres of expertise, healthcare providers and laboratories that operate across borders. Given the low prevalence of rare diseases and in order to cover the thousands of rare diseases that exist, ERNs will be organised into rare disease groups. An initial call for applications for ERNs is scheduled to take place in December 2015. The next step forward is to identify the grouping structure of ERNs and determine how patients can be involved in the ERN decision- and opinion-making process.

Access to orphan medicinal products, treatment and care

Moderated by EURORDIS’s Chief Executive Officer (CEO), Mr Yann Le Cam, and then-EURORDIS Public Affairs Director, Ms Flaminia Macchia, this workshop began with a review by Flaminia of best practices and incentives in orphan medicinal products development. For example, in Italy, Fondo AIFA collects around €45 million a year, which is then invested in development and research of new medicines. This is an independent fund to which pharmaceutical companies have to donate five per cent of their promotional expenditure. Another example comes from France where there is a measure allowing temporary authorisation for medicinal product use without marketing authorisation under exceptional circumstances for severe and life-threatening diseases.

EURORDIS Director for Treatment Information and Access, Mr François Houyiez gave a presentation on how EURORDIS and other patients’ organisations are represented at the European Medicines Agency (EMA) and Health Technology Assessment (HTA) bodies.
the EMA Patients and Consumers’ Working Party (PCWP) and the EMA Committee for Human Medicinal Products (CHMP) agreed on having patient participation in meetings where new treatments are presented to the EMA. This is a pilot procedure where patients are invited to share their views, accompanied by a mentor. However, the difficulty with this project will be making sure that those few patients represent a broader patient community, not only themselves. Relevant tools are therefore being developed. One example is the Protect Project from the Innovative Medicines Initiative (IMI, a public-private partnership) whereby patients are invited to participate in early dialogues with the European Network for Health Technology Assessment’s (EUnetHTA) project on Shaping European Early Dialogue (SEED) and the EMA. The main issues are 1) timing and proceedings: patients, even when trained, have little knowledge of HTA; 2) training and preparation: the European Patient Academy on Therapeutic Innovation (EUPATI) and other initiatives train patients on HTA but most patients who attend SEED/EMA early dialogues have not been trained; 3) travel and accommodation expenses need to be prepaid.

Concerning supply shortages, Mr Houyiez highlighted a few proposals stating that the EMA should create a special department to facilitate prevention, coordination and communication to resolve shortages. In addition, the EMA should create a public catalogue on supply shortages and work more closely with industry to prevent and resolve shortages. Patients and healthcare providers should also be involved in this process. On the other side, public authorities should explore the establishment of buffer stocks and ensure fair distribution of remaining supplies. A monitoring system where stakeholders can report product shortages should also be created.

EHC CEO, Ms Amanda Bok was one of the speakers during the workshop on ‘Centres of expertise’ and moderated one of the many sessions on ‘Patient organisation involvement in the development of care pathways, guidelines and transition from childhood to adult care.’ There was also an update on the RareConnect platform. All presentations and slides are available on www.eurordis.org.

ISTH 2015 Annual Congress

By Brian O’Mahony* and Laura Savini**

On 20-25 June, the International Society for Thrombosis and Haemostasis held its Annual Congress in Toronto, Canada. Brian O’Mahony, President of the European Haemophilia Consortium (EHC), attended the event and gives us his take-away messages on the presentations made during the congress.

*When to start prophylaxis*

Prof Kathelijn Fischer from the University Medical Centre in Utrecht gave a presentation on when to start prophylaxis. She noted that prophylaxis should be started after the first joint bleed with a weekly infusion that should be increased to two or three times a week within six months. Whenever a patient presents a spontaneous significant bleed, physicians should always consider intensifying the prophylactic treatment. Prof Fischer noted that it is important for parents to maintain a detailed diary and for the physician to
review it every three months. With regards to novel treatment and in particular extended half-life products, these may affect the frequency of infusion but not when to start prophylaxis. Prof Fischer also noted that when starting infusions once a week, only 35 per cent of patients will need a central venous access device, which should be removed ideally by the age of six.

Bleeding Scores

Dr Maria Elisa Mancuso from the University of Milan talked about bleeding scores for severe haemophilia and stated that a patient with a bleeding score over three should be considered severe. She noted that the following episodes are worth a score of two:

- A first spontaneous bleed before the age of six months;
- A first spontaneous bleed before the age of two years;
- Ten bleeds per year while being treated on-demand.

She also noted that spontaneous subcutaneous haematomas are worth a score of one.

Mild haemophilia in carriers

Prof Michael Makris from the University of Sheffield and a member of the EHC Medical Advisory Group gave a talk on mild haemophilia. Prof Makris noted that carriers with less than 40 per cent factor should be considered as having mild haemophilia.

FIX deficiency

Dr Elena Santagostino from the University of Milan presented on coagulation factor IX (FIX) deficiency. She noted that severe gene defects are less common than with FVIII (less than 20 per cent in FIX compared to over 60 per cent in FVIII). However, it appears that antigen levels are more detectable. The inhibitor rate with FIX use is similar to that of FVIII if a large deletion is present and she added that in this regard there is no race effect. Furthermore, Prof Santagostino noted that FIX deficiency has a lower prevalence of nonsense mutations (only six per cent compared to 30 per cent in FVIII deficiency). She also stated that anaphylactic reactions to FIX infusions are ten times more common compared to FVIII even though the factor use is lower than with FVIII, which is surprising considering two international units (IU) of FIX are needed to achieve the same effect as one IU of FVIII.
Dr Santagostino also noted that prophylactic treatment is more common for FVIII deficiency than for FIX. For example, in Canada only 32 per cent of people with haemophilia B are on prophylaxis compared to 69 per cent of people with haemophilia A. Currently, there are no specific clinical trials for FIX prophylaxis. Finally, joint replacement in people with haemophilia A is 2.65 times more common than in people with haemophilia B.

Dr Santagostino concluded her talk by comparing on-demand treatment in patients with haemophilia A and B. In people with haemophilia B, the median consumption of 320 IU of FIX per kg per year results in four joint bleeds per year and a Haemophilia Joint Health Score (HJHS) of three. On the other hand, people with haemophilia A with on-demand treatment and a median consumption of 902 IU FVIII per year will experience 11.2 joint bleeds per year with a HJHS of 17. However, the evidence is so far insufficient to recommend different treatment protocols for FIX compared to FVIII use. For example data from the PedNet Haemophilia Registry shows that in 582 previously untreated patients (PUPs) with FVIII deficiency and 76 PUPs with FIX deficiency there is no difference in bleeding rate. This indicates great variation in FIX deficiency and a need for individualised treatment.

**True incidence of inhibitors**

Dr Michael Soucie from the US Centres for Disease Control and Prevention talked about inhibitor incidence rate and the difference in inhibitor development in mild and moderate haemophilia compared to severe haemophilia. Whereas the risk of inhibitor development in severe haemophilia patients is most pronounced in the first 50 exposure days, in patients with mild and moderate haemophilia the risk persists for up to 100 and even 200 exposures, with 16 per cent of treated patients developing inhibitors. This difference is mainly explained by the longer time intervals between each infusion.

In 2012, four cases were reported in the United States of people with mild haemophilia developing inhibitors. They all had lengthy hospitalisations and intensive factor treatment. The key message from this presentation was that all patients with haemophilia, regardless of their severity, are at risk of developing inhibitors and therefore regular screening is needed. Finally, Dr Soucie noted that there was much variability in testing where up to 32 per cent of tests can result in false positives and up to five per cent of tests can result in false negatives.

**Extended half-life factor products**

Dr Guy Young from the Haemophilia Comprehensive Care Centre in Los Angeles moderated a question and answers (Q&A) session on extended half-life factor products.

The first question for debate was: ‘Would you give extended half-life factor to PUPs?’

Prof Fischer answered that she would prescribe the treatment only to a selected population who could benefit from the lower frequency in infusions such as patients with poor venous access and low weight, so as to contain the cost increment.

Prof Gili Kenet from Tel Aviv University said that she would not prescribe extended half-life products as there is no data yet and studies in PUPs are ongoing.

Prof Roshni Kulkarni from Michigan State University noted that she would only prescribe extended half-life products in the context of a clinical trial as there is not enough data to support prescription outside a research environment.

Prof Flora Peyvandi from the University of Milan commented that companies should make their clinical trial data more widely available and this would provide clinical practitioners with
more guidance on whether to use the novel treatments. The EHC has been pursuing this point and has sent all companies developing novel products an appeal letter calling for such increased data-sharing.

Dr Alfonso Iorio from McMaster University noted that families should be informed so they can make an informed consent on whether to use the product or not, however there is currently not enough evidence to make this choice.

Dr Young concluded the discussions by stating that he would not yet prescribe these products because there is not enough information and he agreed with his colleagues by saying that he would only prescribe it in the context of a clinical trial. Furthermore, he noted that there is a lack of clear data on inhibitor incidence and that current trials will take several years to be completed.

There was informal information mentioned during the event that alluded to the fact that some of the extended half-life products resulted in inhibitor development; however, this has not yet been published.

The next question was: ‘If you had to prescribe extended half-life products, what criteria would you use to select people with haemophilia to use these products?’

The answer most commonly agreed upon was to try to move patients who are treated on demand towards a prophylactic regimen. Dr Steven Pipe from the University of Michigan noted that he would select patients that were non-adherent with current treatment, those who were suffering from synovitis with breakthrough bleeds and toddlers.

Prof Kulkarni noted that she would switch people who cannot easily access the haemophilia centre, those who would want their central venous access device removed and haemophilia B patients.

Another question in the panel discussion was about the dosage to use for people on prophylaxis. Most treaters on the panel responded that they would use the same as currently used for both bleeds and prophylaxis. Respondents also noted that they would not expose patients to two different products.

The final question was: ‘Would you treat patients who had developed inhibitors and gone through immune therapy induction with extended half-life products?’

Panellists were asked whether they would treat these patients with longer-acting products. Answers were mixed with some physicians indicating that they would not prescribe longer-acting products as they have not been administered to this category of patients in clinical trials. Others responded that they would as there is no evidence that switching to an extended half-life product would cause inhibitors.

* Brian O’Mahony is the President of the European Haemophilia Consortium (EHC)

** Laura Savini is the EHC’s Communication and Public Policy Officer
EHC attends IPFA/ PEI Workshop on blood safety

By Laura Savini, EHC Communication and Public Policy Officer

Each year the International Plasma Fractionation Association (IPFA) and the Paul-Ehrlich-Institut (PEI) jointly organise a workshop on surveillance and screening of blood-borne pathogens. During this event, professionals working in the area of blood collection and plasma fractionation meet to discuss the latest updates with regards to safety in the area of transfusion, a topic close to the haemophilia community’s heart. This year’s meeting took place in rainy Prague. Laura Savini reports on the two-day event.

Transfusional medicine (the area of medicines that deals with blood collection and the safe use of blood for transfusion) is a delicate balancing exercise. On the one hand there are patients that rely on receiving blood transfusions to overcome illnesses and whose lives may depend on the availability of safe blood components while, on the other hand, there are blood donors, healthy and willing individuals, looking to make a positive contribution towards improving the life of suffering strangers. Blood collection and transfusions are carried out in an ever changing environment where pathogens (known and unknown) lurk in the bloodstream of seemingly healthy individuals. Not only can these pathogens pose a health threat in the recipient but can also be difficult to identify and to assess. For example, on the one hand they could be harmless in healthy individuals but lethal in individuals who have a compromised immune system (e.g. hepatitis E). Other pathogens can be highly infectious and cause severe illness when transmitted by a vector such as a mosquito (e.g. chikungunya and dengue fever) but are much less infectious and will not develop into illness when transmitted by blood transfusion, even though the viral load is much higher.

In other cases, blood collectors will be faced with the difficult choice to establish how much risk is too much risk. For example, during the conference we heard both from presentations and discussions of two examples where almost all of the donor population had been infected by an infectious disease and the collectors had to decide whether to take the risk and collect blood hoping that it will still be beneficial to patients or let the blood supply run dry and import blood from another country (which is delicate due to the fragile nature of blood components), risking being unable to supply patients. The first scenario took place in West African countries during the Ebola outbreak where the Blood Centres of America launched a project to recover convalescent plasma from Ebola survivors to treat Ebola patients. In this case, the blood collectors had to loosen donor selection criteria with regard to malaria. In fact, in West African countries most of the adult population has at some point suffered from malaria, including Ebola survivors. The second scenario took place in Puerto Rico during a chikungunya outbreak where blood collectors decided to stop all blood collection activities for the duration of the outbreak.

This shows the ongoing balance that blood collectors need to reach with collecting enough blood for those in need and ensuring that the blood will not be more harmful than beneficial. This is not an easy task especially when considering that epidemiology of infectious diseases varies across countries (which may impact the level of acceptable risk as shown above) and is increasingly changing due to climate change, societal attitudes and increasing travel. All of
these factors contribute to the emergence of new pathogens and with technological advances new pathogens that have been present all along are discovered with, so far, little knowledge on their impact on recipients.

It was then with no surprise then that a good proportion of the workshop was devoted to learning more on how to assess and manage this risk. The subject was covered extensively and looked at how to develop risk management strategies, devise effective algorithms that would detect the pathogen’s presence in the blood and determine when best to use inactivation techniques that would eliminate pathogens while preserving beneficial qualities of the blood. This also included a presentation on the Risk Based Decision Making Model developed by the Alliance of Blood Operators, which you can further read about on pg 44. The workshop also had some interesting discussions regarding donors’ deferral criteria. For instance, it seems that a simplified and easy to understand Donor History Questionnaire (DHQ - i.e. the standardised questionnaire that is filled in by applying donors before the blood donation) that can be filled in and discussed in a private, non-judgemental environment will most likely promote self-reporting. Another factor that will help with disclosure is the reduction in deferral times. For instance, we heard from an example from Australia where deferral for males who have sex with males (MSM) went from lifetime to twelve months. It was shown that the HIV transmission in blood transfusion did not increase after the deferral was shortened. Australian regulators are now considering whether this period could be shortened to six months. They believe that if donors are educated and understand the risks, they will most likely comply with the donor deferral criteria. Test seeking behaviours in blood donors was also discussed, although it seems that these are in the minority and the figures vary depending on each country’s availability, cost and experience of undergoing tests for HIV and other sexually transmitted diseases. (Regarding MSM and blood donation we refer readers to the EHC’s position statement published on our website).

The talks also looked at the financial aspect and the cost-effectiveness of all of these safety measures. For instance, we learned that the DHQ was found not cost effective according to a Dutch study, however this procedure is maintained because it is seen as an important tool for self-selection and because of the value that society perceives it adds to the safety of transfusions.

Dr Jay Epstein, Director of the US Food and Drug Administration (FDA) Office for Blood Research and Review, provided a very comprehensive final talk in which he stated that despite blood collectors taking many safeguards such as the DHQ, laboratory tests, donor deferral based on their health and risk behaviours, using quarantine control and pathogen reduction techniques there is unfortunately no zero per cent risk when it comes to blood transfusion. The role of regulators, such as the FDA, is to ensure that blood collections move as close as possible towards the zero per cent risk. Unfortunately this is most likely impossible due to continuous emerging health threats. He also reiterated what was mentioned by the Dutch study, and explained that in the United States the cost per Quality Adjusted Life Years (QALY) for blood safety is close to one million US dollars. Again this is justified by the public perception of the value of blood transfusion’s safety, where even a single contamination is seen as an unacceptable risk.

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In conclusion, although the event was highly technical and aimed at professionals working in the area of blood collection, transfusion and plasma fractionation, it was an interesting opportunity to delve into the complexities and challenges present in the area of blood transfusion. This workshop gave much insight into how the risks associated with blood transfusion are approached and managed and this is of great value particularly for recipients of blood transfusions.

The IPFA organises several workshops a year on blood safety in different world regions. To find out where one of their future events is located, please consult the calendar of events at the end of the newsletter.

Risk-Based Decision-Making for Blood Safety

By the Alliance of Blood Operators

Last year the EHC took part in the consultation process organised by the Alliance of Blood Operators (ABO – see EHC Newsletter August 2014) on their development for a Risk-Based Decision Model (RBDM) Framework for blood safety. The framework has now been finalised. Here is a brief summary of the project in which the EHC took part.

When making decisions about blood safety, numerous factors need to be taken into account, such as risks, benefits, costs, ethical issues and stakeholder perspectives. The ABO, an international body composed of blood service organisations from around the world, has developed a unique framework to help blood service operators streamline this process and make responsible decisions that lead to the greatest benefit.

The RBDM Framework for Blood Safety was initiated by an ABO-sponsored International Consensus Conference on Risk-Based Decision Making for Blood Safety held in 2010. The participants agreed that pursuing blood safety risk reduction at any cost is not sustainable. Instead the well-being of transfusion recipients is central to any recommendations to improve blood safety decision-making.

Based on recommendations drawn from this conference, the ABO launched an international initiative to create the RBDM Framework, with the goal of improving decision making, facilitating responses proportional to risk, ensuring evidence-based decisions, increasing trust in investment decisions and allowing for the redirection of resources to improve effectiveness across the blood sector.

This project was a two-year undertaking that included environmental scanning, customization to the blood safety context, wide stakeholder consultation, scenario-based feasibility testing, and peer review. The result of this two-year effort is the finalised first edition of the RBDM framework, which is available on the ABO website.

The development of the framework involved an iterative and comprehensive, multi-platform stakeholder consultation process. The result is a tool for ABO blood operators to optimise the safety of the blood supply, as well as the trust in the RBDM by the stakeholder groups, while taking into account contextual factors that affect blood risk. The integrated stakeholder engagement-based approach allowed ABO to successfully engage a range of diverse national and international stakeholders, which included patients and patient organisation representatives. This engagement aided the development and testing of a complex and technical framework that promises to provide a pragmatic and principled approach to
responsibly manage risk in the blood system. An important face-to-face consultation was held with the Platform of Plasma Protein Users (PLUS)\(^7\), a group which represents organisations of patients with treatable rare diseases, linked by common therapies that use products manufactured from human plasma. The insights gathered from the session were extremely valuable in guiding both the content and the process of stakeholder involvement in decision-making.

Another consultation method used was a broad online consultation called Choicebook, a tool for gathering qualitative feedback on specific issues. More than 300 stakeholders, including patients and patient organisations in multiple ABO member countries were invited to review the framework and then answer a series of closed and open-ended questions about the policy foundations, its four-step process and the tools it offers. Stakeholder responses were used to further refine the risk-based decision-making process.

Stakeholder feedback will continue to play an important role in moving the initiative forward since the framework will be continuously improved as we gain experience from applying it to various risk issues. The RBDM framework is also being converted into an interactive, multimedia tool that will be available on the ABO website. This online tool will permit the user to define a unique path through the content, creating a tailored approach to their decision-making process.

\emph{If you would like more information, please contact the ABO Secretariat at abosecretariat@redcrossblood.org.au}

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\(^7\) The EHC is a founding member of PLUS.
Announcements

EHC 2015 Calendar of Events

Sep 11-13    HTA and Economics Workshop - open to NMOs only
             St Petersburg, Russian Federation

Oct 1-2       Pilot Leadership Conference - open to NMOs only
             Belgrade, Serbia

Oct 2-3       EHC Annual Conference - open to all
             Belgrade, Serbia

Oct 4         EHC Annual General Assembly - open to NMOs only
             Belgrade, Serbia

Nov 20-22     New Technologies Workshop - open to NMOs and selected participants
             London, United Kingdom

Nov 30        Round Table on 'Mild and Moderate Haemophilia' - open to NMOs and selected
             participants
             Brussels, Belgium

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

Other Events

Sep 28-29    IPFA /BCA 2nd Global Symposium on ‘The Future for Blood and Plasma Donations’
             Fort Worth (Texas), USA – More information at http://bit.ly/1NZnCec

Oct 6        European Commission workshop "Access to and Uptake of Biosimilars"

Oct 22-23    WFH Global Forum on Research and Treatment Products for Bleeding Disorders

Nov 5-6      International Primary Immunodeficiency Congress

Nov 24 – 25  EPF Regional Advocacy Seminar - Nordic Countries (open to EHC NMOs from
             Denmark, Finland, Iceland, Norway and Sweden)

Dec 1-2       IPFA Workshop on Improving Access to Plasma and Plasma Products in the Southern
             African Region
             Stellenbosch (Cape Town), South Africa – More information at http://bit.ly/1JvK5Pr

Dec 5-6       ESTM Course on: ‘Learning the best ways for caring for blood donor: the significance
             of this for safer blood and better European Transfusion Medicine’
Publications

The Journal of Haemophilia Practice

The Journal of Haemophilia Practice (JHP) is a new online journal focusing on the care of people living with bleeding disorders.

JHP is published by Haemnet, an independent UK-registered charity that supports education and research for people affected by bleeding disorders and the professionals who manage them. Haemnet believes that sharing experience benefits everyone. Therefore:

- All submitted articles will be reviewed by members of our editorial board and will be accepted if our reviewers feel they offer valuable lessons for others in clinical practice
- Articles will never be rejected simply because of the way they are written; we will copy-edit articles to ensure that your message is conveyed.
- All published articles are freely available to download from our website, by anyone who completes the journal registration form.

JHP is aimed at all members of the haemophilia multi-disciplinary care team, but offers a particular platform for nurses and physiotherapists to share their work and experience. To this end, we positively encourage submission of small scale studies, non-traditional research and case studies from across the world. Although it will be some time before we are listed in MedLine, articles published in the journal are listed on the CrossRef database.

The journal is edited by Dr Kate Khair, Nurse Consultant at Great Ormond Street Hospital in London. It is supported by charitable donations from four companies active in bleeding disorders: Octapharma, Sobi, Grifols and Pfizer. In order to facilitate wider sharing of knowledge and experience about bleeding disorders, we publish under a Creative Commons licence. For more information, please visit the journal’s website at www.haemjournal.com

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 August and includes topics such as:

- Coverage of the 50th Conference of the European Association for the Study of the Liver (EASL)
- New treatment guidelines in hepatitis C
- The different stages of liver damage
- A treatment update

The magazine can be consulted freely and online on the IHS website.

WFH welcomes new CEO

The World Federation of Hemophilia (WFH) has announced the appointment of Alain Baumann as WFH CEO. Mr Baumann brings to this position over 35 years of professional international experience, with a proven track record in non-profit organisations, as well as in the business healthcare sector. Alain Baumann officially began with the WFH on August 3, 2015. The EHC wishes Mr Baumann a warm welcome and much success in his new position!
## EHC Conference 2015 – Final Programme
### 2-4 October 2015, Belgrade Serbia

**Friday 2 October**

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<tr>
<td>09.00-19.00</td>
<td>Exhibition &amp; Poster Display</td>
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<tr>
<td>12.00-13.00</td>
<td>Buffet Lunch</td>
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<tr>
<td>13.00-13.40</td>
<td><strong>Haemophilia in Serbia</strong></td>
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<tr>
<td></td>
<td>Chair: Dr Danijela Mikovic</td>
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<tr>
<td>13.00-13.20</td>
<td>Comprehensive care and inhibitor treatment</td>
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<td></td>
<td><strong>Prof Predrag Miljic</strong></td>
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<tr>
<td>13.20-13.40</td>
<td>The rarer bleeding disorders in Serbia and Europe</td>
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<td></td>
<td><strong>Dr Danijela Mikovic</strong></td>
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<tr>
<td>13.40-14.00</td>
<td>Gene therapy</td>
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<td></td>
<td><strong>Prof Flora Peyvandi</strong></td>
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<tr>
<td>14.00-15.00</td>
<td>Women and bleeding disorders</td>
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<td></td>
<td>Chair: Prof Angelika Batorova</td>
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<tr>
<td>14.00-14.30</td>
<td>Genetic and bleeding risk in carriers of haemophilia: diagnosis and care</td>
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<td></td>
<td><strong>Dr Roseline d'Oiron</strong></td>
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<tr>
<td>14.30-15.00</td>
<td>Pregnancy and menorrhagia</td>
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<td></td>
<td><strong>Prof Rezan Khadir</strong></td>
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<tr>
<td>15.00-15.30</td>
<td>Tea/Coffee &amp; Poster Session – Exhibition Area</td>
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<tr>
<td>15.30-17.00</td>
<td><strong>Complications in Haemophilia</strong></td>
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<td>Chair: Prof Paul Giangrande</td>
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<tr>
<td>15.30-16.00</td>
<td>Thrombosis in bleeding disorders</td>
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<td></td>
<td><strong>Prof Philippe De Moerloose</strong></td>
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<tr>
<td>16.00-16.30</td>
<td>Ageing and cardio-vascular health</td>
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<td></td>
<td><strong>Prof Pier Mannucci</strong></td>
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<td>Time</td>
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| 16.30-17.00  | Orthopaedic surgery  
               Dr Nicholas Goddard                                                   |
| 17.00-17.30  | Tea/Coffee & Poster Session – Exhibition Area                          |
| 17.30-19.00  | Youth Symposium sponsored by Sobi                                       |
| 19.30-21.00  | Welcome Reception and Buffet Supper  
   *Brian O’Mahony, EHC President*  
   *Vladimir Ilijin, Hemophilia Society of Serbia*  
   *Minister of Health or Mayor*               |

**Saturday 3 October**

<table>
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<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>Pharmaceutical Symposium 2 – sponsored by Pfizer</td>
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</table>
| 10.00-11.00  | Inhibitors  
               Chair: Prof Mike Makris                                               |
| 10.00-10.30  | Management of inhibitors  
               *Prof Mike Makris*                                                    |
| 10.30-11.00  | Risk factors  
               *Prof Jan Astermark (TBC)*                                             |
| 11.00-11.30  | Tea/Coffee & Poster Session – Exhibition Area                         |
| 11.30-13.00  | Pharmaceutical Symposium 3 – sponsored by Baxalta                        |
| 13.00-14.00  | Buffet Lunch – Exhibition Area                                         |
| 14.00-16.00  | Long-acting factors  
               Chair: Prof Flora Peyvandi                                               |
| 14.00-14.20  | Clinical experience  
               *Prof Manuel Carcao*                                                   |
| 14.20-14.40  | Measuring lab assays  
               *Prof Jovan Antovic*                                                   |
<p>| 14.40-15.00  | PK-guided treatment                                                    |</p>
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<tbody>
<tr>
<td>15:00-15:20</td>
<td>Prof Paul Giangrande: Post-marketing surveillance and failures</td>
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<tr>
<td>15:20-15:40</td>
<td>Prof Flora Peyvandi: Economics</td>
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<tr>
<td>15:40-16:00</td>
<td>Brian O’Mahony: Discussion</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>Pharmaceutical Symposium 4 – sponsored by Novo Nordisk</td>
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<td>19:00-19:30</td>
<td>Coach Departure for City Tour</td>
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<td>20:00-24:00</td>
<td>Conference Dinner</td>
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**Sunday 4 October**

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<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>09:00-13:00</td>
<td>Exhibition; Poster Display; Hospitality Desk</td>
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<tr>
<td>09:00-11:00</td>
<td>EHC General Assembly (open to NMOs only)</td>
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<tr>
<td>11:00-11:30</td>
<td>Tea/Coffee – Exhibition Area</td>
</tr>
<tr>
<td>11:30-13:00</td>
<td>EHC General Assembly (open to NMOs only)</td>
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