



EHC NEWSLETTER

December 2017

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EHC Newsletter December 2017

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President and CEO Report

The end of the year is always a time of reflection. What change did we accomplish? Did we address the challenges we set out to tackle in the beginning? Writing our goals on the pages of the first 2017



Brian O'Mahony
EHC President



Amanda Bok
EHC CEO

EHC newsletter (see April issue), we aimed higher than before; although witnessing significant improvements in haemophilia treatment and care, we knew that this is no time to slow down. Our community's history has already been written, but the future is ours yet to build.

PARTNERS

With this in mind, a large part of our focus was dedicated to increasing access to haemophilia treatment in countries that still provide little or no such therapies to their haemophilia population. Though the framework was laid

out in previous years, 2017 saw the significant launch of our Procurement of Affordable Replacement Therapies - Network of European Relevant Stakeholders, PARTNERS, programme (see pg 23). Developed as an approach to create a sustainable national procurement model for haemophilia A and B treatment products, the programme involves multiple stakeholders who will work with national health care systems in order to enable countries to provide adequate levels of factor concentrates and to formally involve haemophilia clinicians and patient representatives in national procurement processes to do so. This may translate into increased access to vital treatment for over 5,000 people with haemophilia in those Central and Eastern European countries eligible for the programme. In addition to having conducted nine successful country visits in 2017, we are also delighted to announce that Kedrion Biopharma and Swedish Orphan Biovitrum (Sobi) have now signed the full memorandum of understanding (see press release on pg 25).

Events

Tenders and Procurement Workshop

The importance of involving patient representatives and haemophilia clinicians in the procurement of treatment products is also highlighted in our series of economic workshops on *Tenders and Procurement*. The second such workshop was held in September in Sofia, Bulgaria (see pg 9) and gathered 20 representatives from our National Member Organisations (NMOs) for training on becoming involved in national procurement systems for haemophilia treatment products. Our belief and experience



are that not only does such inclusion lead to better and more efficacious outcomes, but that it also forms the base from which better quality of life for people with haemophilia starts to become a reality.

European Inhibitor Network Summit and workshop

Solidifying the work of the EHC European Inhibitor Network (EIN) and widening its impact, the Inhibitor Working Group in October held the first in a series of annual small workshops on better inclusion of people with haemophilia and inhibitors (PWI) in their NMOs (see pg 11). In December, the EHC held its



EHC Inhibitor Summit 2017

second annual Inhibitor Summit in Barretstown, Ireland, where we were again moved and motivated by the stories and bonding that took place between the 126 patients, families and caregivers in attendance. With half of the participants as first-time Summiteers and the other half as returnees, we were pleased to see a strong and vibrant community forming and building their voices.

A personal story from the Summit will follow in our April newsletter; in the meantime, we invite readers to see the photos from the event on our Facebook page and take this opportunity to thank the sponsors - Alnylam, Roche and Shire - who made the 2017 EIN programme possible!

EHC Conference

We were delighted to see our largest-ever attendance, over 325 participants, at our 2017 EHC Conference in Vilnius this year (see pg 13) not just because of the number, but because it displays a deeper interest, reach and collaboration of and between our NMOs, thereby strengthening our broad efforts. Alongside the latest updates in current and upcoming novel haemophilia treatment, symposia and discussions, the Conference also provided a stage from which to launch the EHC's new online platform for people with haemophilia and inhibitors and to premiere our new film, *Inhibitor Stories*, fruit of another moving collaboration with Goran Kapetanovic (and now available at www.ehc.eu/community), which is why we were also pleased to surprise Goran with a "Best Director in a Bleeding Role" EHC-Oscar at the event!

In the meantime, preparations have already started for the 2018 EHC Conference, which will be co-hosted by the Azeri National Member Organisation with a small change - namely that the Conference will be held in Brussels, Belgium, due to visa issues. While we cannot take our broad community to Baku just yet, we look forward to Baku coming to us. Details will follow in the New Year!

New Technologies Workshop

Finally, we held our fourth New Technologies workshop in November and, with all the new pipeline work currently being carried out, had much to cover in the programme. Hitting our highest attendance ever, with more than 50 participants, we examined the shifting landscape of haemophilia treatment and discussed how to prepare the European patient and clinician communities for this exciting future (see pg 19).

Round Table

Rounding off the year, our last Round Table of the year (see pg 21), held in November and chaired by our MAG member Mike Makris, focused on the usage and measurement of extended half-life and novel products coming down the line. Through a series of comprehensive talks, we were able to dive deeper into what it really means to make big shifts in treatment paradigms - from laboratory to regulatory implications - and give our broad community one clear take away for what will be required to navigate the brave new world ahead: 'education, education, education!'

Collaborations old and new

This quarter we thank all outgoing members of our Inhibitor-, and Women and Bleeding Disorders-, Working Groups for all of their hard work and commitment, and we welcome the new members with whom we look forward to collaborating in the coming years!

We also sincerely thank each and every Member of the European Parliament (MEP) who has supported our work through the past years in our informal group. Last month we officially formalised this group and very much look forward to collaborating more deeply and strategically with the new MEP Group on Rare Bleeding Disorders in the years to come!

Finally, we welcome new strategic collaborations with members of our new Medical and Scientific Advisory Group (MASAG, see pg 29) who held their first face-to-face meeting in October, and with the EASL Foundation, with whom we met in October and agreed on a Plan to Eradicate Hepatitis C from the European haemophilia population by 2022 (see pg 18).

The future is bright.

So goes the popular saying. For the haemophilia community, it certainly seems that way. Promising novel therapies are set to enter the market and others will move further down the pipeline in 2018. To help guide our NMOs the EHC will issue the first in a new series of 'New Product Quarterlies' in the beginning of next year. More members of our broad patient community are gaining their voice. With the help of our former Women and Bleeding Disorders Working Group (now a Committee), we launched a survey from which we look forward to sharing results next year - most certainly, they will help strengthen the voices of many who have until now remained mostly unheard. Our community with inhibitors is also better represented than ever before and with our submission for publication of the 'European Principles of Inhibitor Management' we hope that a harmonized approach to their treatment will soon follow. The EHC's external consultation process has drawn to a close and resulted in rich feedback, which will help us elaborate a new Strategic Plan 2018-2022. While the Commission Expert Group for Rare Diseases, on which we were represented, has ended its mandate and will not be renewed, the work of the European Reference Networks (ERNs) is taking off; the EHC is playing an active role in the field of guidelines and best practices and the area of ERNs will only continue to grow.

Saying hello...

And speaking of growth, we welcomed new life into the EHC family with the birth of a beautiful baby girl to Laura Savini, our Public Policy Officer, and we are sending Kristine Jansone, our Inhibitor Program Manager, off into maternity leave in expectation of her baby-boy-to-be, whose birth we expect in January. Coming on board to help us in Kristine's absence is Fiona Brennan, who had recently left the Irish Haemophilia Society and brings to the EHC years of community-building experience. We are delighted to have her joining our team and invite you all to give her a warm welcome. Both Laura and Kristine will be back in early/late May and until then, we wish them well!

...and saying goodbye.

It is not just our mothers-to-be and the year that we send off. With great sadness we also say goodbye to a dedicated member of our community, President of the Serbian Haemophilia Society Vladimir Ilijin, who passed away suddenly this month, but who leaves a great foot print not just in his home country but in Europe and the world, and whom we honour for his passion and dedication (see pg 44).

With this last note from us for this year, we thank you: for the partnership, for the perseverance, for the pleasure of working on behalf of this great community. We wish you well deserved rest over the holidays and a Happy and Healthy New Year!

EHC NEWS

Comprehensive care in haemophilia: pain management

Interview with Professor James Elander; Interview taken by Raia Mihaylova, EHC Communications Officer

Haemophilia is a rare bleeding disorder but treating it requires a comprehensive approach by professionals in other medical areas as well – physiotherapists, haematologists, inhibitor specialists and orthopaedics, to name a few. Though joint issues fall under orthopaedics, they are also the source of a different concept in need of attention – pain management. Maybe because it is a more personal issue or one of “less importance” compared to other challenges in haemophilia care, the topic is often put on the back burner, causing people with haemophilia to deal with pain in isolation. Professor James Elander, a health psychologist, has long been involved in working with people with haemophilia in Europe and joint pain, and notes that this is an issue shared by most. With this interview, he hopes to provide ideas on how to better address joint pain, both by acknowledging and managing it.



Professor James Elander

Can you briefly introduce yourself and your area of work? How did you get involved in this field?

I am a health psychologist at the University of Derby. Health psychologist – that means someone who is interested in looking at the psychological and behavioral factors that contribute to a person’s physical health; it covers quite a wide range of things. I have been at the University of Derby for about five years but it was before coming here that I began my work in haemophilia. That interest started about 15-20 years ago and the way it began gives a nice example of the importance of student projects. I got involved working with haemophilia through a student who came to me, she was in her final year and doing her undergraduate project. She said

she wanted to do something related to haemophilia because she was interested in the topic. I didn’t know too much about it at that time except that it was a bleeding disorder and I asked her “is there pain involved in haemophilia, is it a painful condition?” So, I was working with her and it really helped me to understand the painful aspects of haemophilia. She developed a small study about pain coping; I began developing further studies from this and that is how it all started.

So you got involved by chance – what surprised you about haemophilia along the way?

I wasn’t aware that it was such a painful condition, especially on the joints. I knew it was a bleeding disorder, inherited mainly by males from female carriers, as well as some of the traditional history of haemophilia – how it impacted the royal families. I knew that kind of history, not the scientific history. I gradually began learning about it as a pain condition and how the joints are affected by bleeds. Blood vessels in the joints can get pinched, causing the joint space to fill with blood. When that happens repeatedly over time, it leads to atrophy. One aspect that has surprised me since then was learning about the effect of factor concentrates and how, when administered prophylactically, it makes a really big difference. With prophylaxis, pain has become less of a problem because people are more able to avoid bleeding episodes. But as we know, prophylactic treatment is really expensive. In poorer countries, it is less available and I think we are going to see more of a divide between people with haemophilia in rich countries, where they can afford quality care and they will have less pain, and

people with haemophilia in poorer countries, where they are going to continue experiencing a lot of joint pain and a reduced quality of life. And that wide difference to me is very sad.

From personal observations, it seems that joint pain and overall experience with pain by haemophilia patients is much more widely talked about and addressed in the U.S. than in Europe. You work closely on the topic, what are your views?

I have read a lot of research from North America and I have had some correspondence through email with American researchers. I wouldn't myself suggest that they are ahead of Europe in terms of focusing on pain and problems of pain management in haemophilia care. There certainly is a lot of evidence from the U.S. that people with haemophilia don't feel that their pain is properly addressed. Having said that, in Europe, there is also still a lot of progress that needs to be made in terms of producing guidelines. These are important because they would help treatment centres help patients to cope better with their pain and manage it better.

What are some of the pain coping mechanisms you would suggest?

It is hard to say "here is the key coping strategy" that will work for most people. People are different and different things will work for them. In the work that I have done, the key thing that I try to help people do is to reflect more on their pain, be observant of it, notice the things that they can do and what makes their pain worse or better.

There are many helpful mindful strategies, like thinking differently, distracting yourself from the pain; it is more about the motivational side of things. Acceptance of pain is an interesting concept. In some ways, this is a misleading term because it doesn't mean that people should just accept that they are in pain and do nothing about it. It is a kind of movement or a kind of theory to emphasize people's value, to focus on what is important in their lives – their families, their work, their activities, things that they love – and work out ways to keep going with those activities in spite of having a chronic pain condition. It is really about not letting pain take over one's life.

Then, there is, of course, pain medication, but it needs to be used effectively. Because with pain medication, it is all about finding the right balance for each individual; the right balance between getting the benefits of pain medication against avoiding the negative side effects of pain medication – getting addicted or getting to the point where it is actually reducing the person's quality of life.

What are some approaches to self pain management?



Original Article

Motivational and behavioural models of change: A longitudinal analysis of change among men with chronic haemophilia-related joint pain

J. Elander, C. Richardson, J. Morris, G. Robinson, M.B. Schofield

First published: 17 April 2017 Full publication history

Abstract

Summary. Pain coping strategies are important influences on outcomes among people with painful chronic conditions. The pain coping strategies questionnaire (CSQ) was previously adapted for sickle cell disease and haemophilia, but those versions have 80 items, and a briefer version with similar psychometric properties would facilitate research on pain coping. The full-length haemophilia-adapted CSQ, plus measures of pain frequency and intensity, pain acceptance, pain readiness to change, and health-related quality of life were completed by 190 men with haemophilia. Items were selected for a 27-item short form, which was completed 6 months later by 129 (68%) participants. Factor structure, reliability and concurrent validity were the same in the long and short forms. For the short form, internal reliabilities of the three composite scales were 0.86 for negative thoughts, 0.80 for active coping and 0.76 for passive adherence. Test-retest reliabilities were 0.73 for negative thoughts, 0.70 for active coping and 0.64 for passive adherence. Negative thoughts were associated with less readiness to change, less acceptance of pain and more impaired health-related quality of life, whereas active coping was associated with greater readiness to change and more acceptance of pain. The short form is a convenient brief measure of pain coping with good psychometric properties, and could be used to extend research on pain coping in haemophilia.

This study adds longitudinal evidence about sequential change processes; a test of the motivational model of pain self-management; and tests of behavioural versus motivational models of pain acceptance.



A brief haemophilia pain coping questionnaire

J. ELANDER, G. ROBINSON

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and as the title suggests, it features five individuals with haemophilia who share their experience with pain. The idea was to really encourage viewers to see that it is possible to get involved in the management of one's pain and to motivate and inspire people to be more active in that aspect. That is the first step. After that, one can begin looking at more skills-based things, learning techniques that will help, etc.

One thing that is really important and that makes pain in haemophilia different from other chronic pain conditions is the importance of treating acute joint bleeds. If a person has an acute bleed, they need to treat themselves and take some self-management action. They shouldn't be accepting that pain. It is very important to tell the difference between and distinguish acute bleeds from pain caused from past bleeds, so that it can be correctly addressed.

Is addiction to pain medication a problem in Europe?

Yes, it is. There is a big problem, a chronic problem. We see that this is a massive problem in the U.S., there has been massive media coverage lately, showing how much damage it is causing. I think in Europe, we are behind the U.S. but it is still a big problem. It would be interesting to know what proportion of people with haemophilia are addicted to their pain medication. But in general, yes, there is a big problem nationally in Britain and in many other countries with addiction, people are abusing pain medication and taking it out of control. That is something we need to be mindful of and talk about.

What is the line between using pain medication correctly and it becoming an addiction?

Right, how much is too much? How do you tell if a person in pain has a problem with addiction? It really is a question of the balance I mentioned earlier; when the balance is gone, the medication is no longer working well to manage their pain, they are not getting the benefits and maybe the way they are using the pain medication has changed over time. A person might have begun to control his pain but then gradually is maybe taking the medication more and more often, and is finally using it for other reasons, like to avoid feelings of anxiety or depression. So, when I am asked "Am I using too much, what are the signs of addiction?", I think: 'If your pattern of use has changed, if you're using more and you feel like it's not helping you manage your pain as well as it used to, then there probably is a problem there.'

What are some results of your work that you are proud of?

One of the best experiences for me was the filming we talked about earlier, where we produced this video "Five Lives." When it was finished, we sent it to a group of people with haemophilia and we followed them up over time and compared them to a group of people who hadn't received the video. The project showed that people who had received it had improved in their motivation to self-manage their pain. So, it showed me that it could really help some people to start thinking about becoming better at self-management; I was really glad to see that it had started them down the path.

*If you would like to receive a copy of the "Five Lives" DVD, you can write to James at j.elander@derby.ac.uk

Self-management – it starts with willingness to think about pain and what is a proper solution; with willingness to take responsibility about how one manages the pain; willingness to make decisions about pain medication. That is the focus of a DVD that, together with some other colleagues, we produced along with an information leaflet. The DVD is called "Five Lives"

EHC Tenders and Procurement Workshop: bringing the haemophilia voice into the purchasing process of treatment products

Written by Raia Mihaylova, EHC Communications Officer



Participants from 11 countries took part in this year's workshop

A close patient-clinician relationship is at the base of haemophilia care; only when both sides are actively involved in the choosing of an individualised treatment regimen, in education and in communication with the multidisciplinary team, can optimal health results be achieved. One brings knowledge to the table, the other brings experience, and this exchange of information is invaluable to the process.

The same idea applies when it comes to the tender and procurement of haemophilia treatment products. Throughout Europe, more often the purchasing of factor VIII and factor IX concentrates is carried out by national government representatives who are well-acquainted with the technical side of the process, but not with haemophilia itself. As the results of the tender directly impact the availability of treatment, its quality and therefore, the quality of life of people with haemophilia, it is of extreme importance to have patients and clinicians formally included in the decision-making process. This collaborative approach can ensure best tender outcomes.

Inclusion of patient representatives and clinicians in the national tender for factor concentrates is also one of the twelve 2016 Wildbad Kreuth recommendations for the optimal treatment of haemophilia in Europe, which were recently endorsed by the Council of Europe. But for this involvement to be meaningful, preparation is key.

It is with this purpose that from September 15-17, the EHC held the second of a series of workshops on tenders and procurement, which took place in Sofia, Bulgaria. Patient organisation representatives from Albania, Bulgaria, Croatia, Estonia, Greece, Hungary, Israel, Lithuania, Macedonia, Poland and Serbia joined for a two-day training aimed at providing the tools and practical knowledge on how to become more involved in national tenders. The programme covered all relevant concepts, from treatment evolution throughout the years and its safety criteria to the breakdown of tender and procurement systems in Europe.

To put the theoretical information given into practice, participants also took part in a mock tender, including developing treatment selection criteria and scoring factor concentrate products. They were presented with different scenarios and conditions under which treatments were developed and had to make a decision based on which one they thought was the most, safe, efficacious and cost effective, amongst other factors



Dr Glenn Pierce, Board Member of the World Federation of Hemophilia, presented basic concepts in replacement therapy.



The justification for their final choice of treatment product displayed that participants had picked up on what is important to consider when carrying out a tender and how to accomplish best results through collaborative efforts.

The next and final workshop on Tenders and Procurement will take place from September 14-16, 2018.

This workshop was made possible by an educational grant from Pfizer.

European Inhibitor Network (EIN) Workshop 2017

*"Finding ways for better inclusion of PWI in the life of their NMO" – My observations
Written by Jim O'Leary, member of the EHC Inhibitor Working Group (IWG)*

On the 4th of October 2017, the EHC Inhibitor Working Group (IWG) and a number of invited National Member Organisations (NMOs) members descended on Vilnius for the pre-EHC Conference workshop on better inclusion of people with inhibitors (PWI) within the life of their patient organisations. This was the first such workshop we organised and it was a pilot designed to help us decide if this format would be useful and if we should continue with it in the future. We were 15 persons in total, which I initially thought might be a small number but later changed my mind, as it was easier to try different things with a smaller group. The programme was carefully and methodically prepared by Kristine Jansone (EHC), with input from the IWG members. The event lasted a full day; after registration in the morning, the attendees were welcomed on behalf of the EHC by Brian O'Mahony and Amanda Bok. Kristine then explained the elements of the programme and how we would proceed for the rest of the time. We were warmed up with a fun ice breaker of human bingo.



Icebreaker – human bingo



Presenting the needs of people with haemophilia and inhibitors

Later, we moved on to talking about existing good practices from NMOs on different problem areas. Presentations were given by: 1) Carl Frederik (Sweden, IWG) on letters to send to the Haemophilia Treatment Centres (HTC) from the NMO, which can then be given to PWI and allow them to make contact with the NMO and to take advantage of the support, which is available; 2) Mirko Jokic (Serbia, IWG) on special activities and active engagement of PWI by the NMO; 3) Jim O'Leary (Ireland, IWG) on training and self-advocacy of PWI, starting from a basic level of involvement and self-advocacy. The presentations were then discussed by the group to see if

The first full session focused on understanding the specific needs of the participating NMOs in order to be able to better include PWI. I – Jim O'Leary, IWG – gave a short presentation of my needs as a PWI – medical, social, psychological, educational, employment etc. This was followed by a short presentation by Miguel Crato (IWG) on the vice-versa needs of the NMO. Following the presentations, we split into working groups of three to four people and Kristine introduced to us to the concept of a problem tree, which we each used to solve a particular problem from the presentations. This got the groups talking and working as a team, becoming even a bit competitive with one another.



Participants discuss ideas on how to address challenges faced by PWI

these were areas that could be improved in other NMOs and that the participants could address when they returned back home.

In the final session before lunch, Kristine outlined some of the available tools for inclusion, developed by the European Inhibitor Network (EIN). She then presented the Human Rights Based Approach (HRBA) to solving problems by identifying and including all stakeholders to reach a solution. The tools presented in the morning session were an excellent introduction to how the needs of PWI can be better addressed.

After lunch, we returned to the problem tree introduced in the morning session. This time, the method was adapted to produce a solution tree for the problems previously identified. Again, we worked in groups of four to pool our resources and produce the best solution tree. What a competitive lot we are! Each group then presented their solution tree and how they arrived at it.

The final part of the workshop was for conclusions and discussion on further steps we could take. I think it was a very useful workshop that was enjoyed by all and it should be rolled out to a larger audience, perhaps again before next year's EHC Conference or even at the 2018 Inhibitor Summit.



The Inhibitor Working Group, together with participants of the workshop

This workshop was made possible through the support of the EHC European Inhibitor Network by Alnylam, Roche and Shire.



2017 EHC Conference

Written by Raia Mihaylova, EHC Communications Officer

The EHC Conference is one of the largest patient gatherings in Europe and each year aims to provide the latest treatment information and to be a place for exchange of experiences between National Member Organisations (NMOs) and open dialogue between patients and health care experts.

From the 6th-8th of October, more than 300 participants gathered in Vilnius, Lithuania, for three days of scientific lectures, debates and symposia. The coming together of the community always inspires the EHC and its NMOs to continue efforts in trying to assure quality care and full access to treatment for people with haemophilia in all European countries. Experience some of the moments from the EHC 2017 Conference through the highlights below!

The EHC would like to sincerely thank the Lithuanian Haemophilia Society for hosting the EHC Conference and the volunteers for their valuable help! The EHC 2018 Conference will take place on October 5-7 in Brussels, Belgium!

Examples of Presentations



Professor Flora Peyvandi, member of the EHC Medical Advisory Board, on the new advantages and challenges that novel haemophilia therapies will bring. She stressed the importance of educating both physicians and patients on the usage of these treatments.



Director of the Department of Gene Therapy and Regenerative Medicine at the Free University of Brussels, Professor Thierry VandenDriessche, broke down the complexities of gene therapy for haemophilia and discussed where it currently stands.



Professor Cedric Hermans (center), President of EAHD, on whether novel treatment will change the management of inhibitors. "For one, it could achieve an annual bleed rate of 0 in people with inhibitors," said Prof. Hermans.



Session on orthopaedic issues in haemophilia. Physiotherapist Ralf Kalinowski (left) focused on rehabilitation after total hip and knee arthroplasties; Dr Gianluigi Pasta (center) spoke about the importance of early radioactive synovectomy; Nurse Petra Elfvinge displayed the key role nurses play in surgery.

Youth Debate



The Youth Debate, an already well-established tradition of the EHC Conference, once again had young and dynamic voices debating against esteemed figures in the haemophilia community on key, and often controversial, issues. This year, three youth representatives – Robbert-Jan t'Hoen, Valentin Brabete and Tobias Krucker – had ten minutes to argue their positions on a previously assigned topic before their opponents took the stage. Read below what issues were debated, along with Valentin's account of what the experience was like for him.

The EHC Youth Debate was made possible thanks to an educational grant from Sobi.

Debate one: Patients should have the final say in treatment product selection.

“For” argument: Youth representative
Robbert-Jan t’Hoen, Netherlands



“Against” argument: Dr Paul Giangrande, Chair of the EHC Medical Advisory Group.



Debate two: Payers should consider haemophilia a special case.

“For” argument: Youth representative, Valentin Brabete, Romania



“Against” argument: Mark Skinner, member of the Medical and Scientific Advisory Council of the National Federation of Hemophilia



Youth Debate 2017: The personal experience of Valentin Brabete

Taking part in the EHC youth debate during the EHC 2017 Conference held in Vilnius, Lithuania, in October, was definitely a surprising experience for me. I have previously been in front of a large audience, but having to talk about a matter so important to me in front of a knowledgeable audience significantly changed the game. I knew that this debate was intended as an exercise and that everybody has fun practising it, so initially I took it quite lightly. However, the more I thought about it, the more I realised that I most likely will soon enough be in a real-world situation, where I have to argue in front of the payer why they should consider haemophilia to be a special case. At that thought, the stakes completely changed and I started to seriously think about my arguments, what the weak points might be that my opponent could attack, and I tried to foresee and pre-empt that line of argumentation. I tried to do my best and I think I've learnt a lot from this exchange of arguments. What I also reflected on, following the experience, is that when I went up on stage I had no panic regarding the large audience, but I started to fear that whatever I was saying would not be representative of, or could even offend, part of our community. These thoughts really gave me the chills while on stage and I was desperately trying to make eye-contact and get feedback from my peers, while also trying to keep a constant and calm tone. This really made me think of the responsibilities that you actually bear as a representative of the haemophilia community, what its needs, fears, weak and strong points are. When you are up there, it is not just about you, it is about representing our whole community!

Debate three: Pre-natal selection should be available to all in the bleeding disorders community.

“For” argument: Youth representative, Tobias Krucker, Switzerland



“Against” argument: Dr Dan Hart, member of the EHC Medical and Scientific Advisory Board



Premier of *Inhibitor Stories*

We were proud to release the *Inhibitor Stories* documentary, produced by director and film-maker Goran Kapetanovic. His cameras captured the powerful stories of six people with haemophilia and inhibitors from different countries in Europe, highlighting the different outcomes that disparities in access to treatment lead to. This is the second piece of work done by Goran for the EHC, following the release of last year's documentary *Haemophilia Stories* (see www.ehc.eu).



Director Goran Kapetanovic spoke about the experience of filming Inhibitor Stories.

Inhibitor Stories can be viewed at <https://www.ehc.eu/community/>. We sincerely thank everyone who took part and shared their story!

Funding for this film was supported by Novo Nordisk.

The screenshot shows a web browser window with the URL <https://www.ehc.eu/community/>. The page header includes the EHC logo and navigation links for HOME, ABOUT, FOR NATIONAL PATIENT ORGANISATIONS, FOR PATIENTS, FOR PARENTS AND CAREGIVERS, and CHAT ROOM. Below the header is a large, atmospheric photograph of a forest. Overlaid on the image is the title "Inhibitor Stories" and a subtitle "Inhibitor Stories Documentary". A text box below the title reads: "Go inside the realities of day-to-day life of people with haemophilia and inhibitors. Hear their stories and experience what different outcomes disparities in access to treatment throughout Europe lead to." To the right of this text is a white rectangular button with the text "Watch Inhibitor Stories here". At the bottom of the page, there is a taskbar showing various application icons and system status information.

Launch of platform for people with haemophilia and inhibitors

Forming a strong community of people with haemophilia and inhibitors has been one of the main goals of the EHC European Inhibitor Network and the idea behind the

EHC Inhibitor Summits. We wanted to further expand this reach and create a virtual community that can easily connect people with inhibitors year-round.

That is how www.ehc.eu/community was born! The website is a combination of an online inhibitor library and a virtual community, making it one of a kind. Visitors can find a lot of useful information about inhibitors, advice on day-to-day issues, travel and professional guidelines, and of course, speak with other peers facing similar challenges.

The most powerful part on this website is the meeting point, or chat room. Connect with other peers just to talk or to exchange experience and advice. There is also an opportunity to speak with medical professionals and ask questions on a topic of concern in the section “Ask the Expert.”

Head over to www.ehc.eu/community and explore the website! We will be frequently updating content and posting up-to-date information.

Update from the Women and Bleeding Disorders Working Group about the women's booth at the EHC 2017 Conference in Vilnius

Written by Christina Burgess, Member of the EHC Women and Bleeding Disorders Working Group and the EHC Inhibitor Working Group

The Women and Bleeding Disorders Working Group has worked to promote awareness of the many issues experienced by carriers and women with bleeding disorders across Europe. In October this year, the group had a stand at the EHC Conference in Vilnius, Lithuania, in order to keep creating awareness. All National Member Organisations (NMOs) had been invited to share any material they might have about women and bleeding disorders and several NMOs did bring their materials to inspire others.



The Women' booth at the EHC Conference

During the course of the conference numerous attendees visited and availed themselves of information, leaflets and relevant, useful advice. Interest in the work being carried out was shown by both males and females, whether affected with a disorder themselves, or having a partner or daughter who is a carrier or who has a disorder. The stand was also visited by many health care professionals and representatives from pharmaceutical companies who are beginning to recognise that this has been a very under-represented and under-supported cohort until now.

At the stand everyone could bring suggestions and ideas about the focus of future work regarding women and bleeding disorders. Suggestions include better diagnosis, more education, and better access to information. The group is delighted to receive all the suggestions.

A very big “thank you” to the volunteers manning the booth, in particular Yannick Colle, Evelyn Grimberg, Marija Nakeska and Marie Lahn Rømcke who made an extra effort to make this booth such a success!



Meet the members of the newly formed Women and Bleeding Disorders Committee in the next issue of the newsletter, coming out in March of 2018!

New Collaborations

The EASL International Liver Foundation poised to initiate a new collaboration with the European Haemophilia Consortium

The article was written by the CEO of the European Association for the Study of the Liver (EASL) International Liver Foundation, Stefano Gnes and published on <https://easl-ilf.org/news/easl-international-liver-foundation-poised-initiate-new-collaboration-european-haemophilia-consortium/>

A new collaboration between the EASL International Liver Foundation and the European Haemophilia Consortium is expected to take place over the coming months. The two organisations held a consultative meeting in Vilnius, Lithuania, on Friday, October 6th 2017.

Over the course of the meeting the leadership of the two organisations identified a number of areas for potential collaboration, amongst which is the aim to eliminate hepatitis C by 2022.

"The European Haemophilia Consortium represents a very dynamic, resourceful and committed organisation. I am confident they would represent an invaluable collaboration asset to the Foundation," said Professor Massimo Colombo, Chairman of the Foundation.

Exploring the Horizon with the ‘New Technologies in Haemophilia’ Workshop

Written by Raia Mihaylova, EHC Communications Officer

The emergence of novel haemophilia therapies onto the market brings long awaited excitement, hope and... many questions. Will they cause inhibitors, what needs to be considered when making the switch to a new product, how will their long-term safety and efficacy be monitored?



Panel discussion on how patients have been switched to extended half-life products in Europe, with examples from Germany, the UK and Ireland.

achieving a high quality of life and that fear of inhibitor development is often what holds patients back in making the decision to switch to a new product, especially if their current treatment is working well. The main take-away conclusion, however, was that both patients and clinicians still need to be further educated on novel treatments, in order to be able to maximize the benefits this transition will bring.

We invite you to watch the following interviews, which were taken at the workshop and which provide useful information on novel therapies in haemophilia care.



Interview with Dr Pratima Chowdary, Consultant Haematologist, Royal Free Hospital.

Topic: Which parameters need to be considered when evaluating the benefits and long-term efficacy of new clotting factors?

*Click on the image to access the interview or follow this [link](#).



Interview with Dr Alison Dougall, Clinical Consultant at the Dublin Dental University Hospital.

Topic: Game changers in haemophilia dental care.

*Click on the image to access the interview or follow this [link](#).

The New Technologies Workshop was made possible thanks to educational grants from Roche, Shire and Sobi.

EHC Round Table on Clinical Trials on the Usage and Measurement of Extended Half-Life Coagulation Factors and Non-Substitutional Therapies

Written by Yordan Aleksandrov, Consultant, Rohde Public Policy (RPP)

On 28 November 2017, the European Haemophilia Consortium (EHC) held its third Round Table of Stakeholders of the year at the Sofitel Hotel in Brussels, Belgium. The event brought together over 40 participants representing researchers, industry, patients and academia to discuss the usage and measurement of extended half-life coagulation factors and non-substitutional therapies.



The event's agenda, list of speakers and presentations can be consulted on the [EHC website](#). Pictures from the event can be viewed on the [EHC Facebook page](#).

On the Usage and Measurement of Extended Half-Life (EHL) Coagulation Factors and Non-Substitutional Therapies



Dr Roger Schutgens on current use of EHL and non-substitutional therapies

Haemophilia is a rare and congenital bleeding disorder caused by a genetic defect, resulting in a lack of or insufficient coagulation factors VIII or IX in the body. In affected individuals, this causes an inability to clot blood, leading to bleeds in the joints, muscles and soft tissues. If left untreated, this can lead to disability and sometimes death. It is widely agreed that the optimal standard for haemophilia treatment is prophylactic substitution therapy, i.e. the regular infusion of the missing coagulation factor.

Extended half-life (EHL) coagulation factors are clotting factors, which stay in the patient's blood circulation longer than standard clotting factors. This enables more treatment strategies.

Findings and discussions

With bleeding episodes being a reality for people with haemophilia, it is essential to prevent them as much as possible. The optimal standard for haemophilia treatment is prophylactic substitution therapy. However, frequent intravenous infusions are still a burden, while patients still experience bleedings despite prophylaxis. Treatment can be optimized and for this there is a need for other products. While according to studies many patients are ready to switch to EHL products, a reasonable amount have their doubts due to uncertain safety of new products, inhibitors risks, immediate availability of current product, etc.

Assessing value: standard treatment vs novel technologies

When assessing the value of standard treatment against novel technologies, it is crucial to define what value are we looking for. During the EHC Round Table the following concepts were discussed: safety, efficacy, availability, ease of use, lifecycle costs and financial acceptability. Speakers stated that

national procurement of factor concentrates can help to ensure that people with haemophilia, von Willebrand disease, and other inherited bleeding disorders have access to treatment that is not only sufficient in quantity, but also meets the required standards in relation to safety, efficacy and quality.



Professor Cedric Hermans gave an overview of haemophilia and the evolution of its treatment

future is always a risky business. However, it can be anticipated that standard- and EHL clotting factors will still be used routinely. Perhaps in future more than one novel, non-substitutional therapy will be widely used, including drugs targeting physiological anticoagulants. In 10 years' time, haemophilia care will have been transformed in an unprecedented way. Many questions will have to be answered and we have to advocate for the pursuit of these answers, while as a community, people with haemophilia have to get comfortable with the new language of novel therapies and prepare to take firm positions.

Conclusions

The practical switch towards EHL, or indeed consideration of the novel therapies coming down the line, create a brave and very new world. All these choices could become available for patients not only in developed but also in emerging countries. However, and as rightfully pointed out during the presentations: 1) it is always tricky to predict the future and; 2) all these new choices will require significant education on the part of all stakeholders. The only certain thing is that the future will be different and both the patient and the clinician community must learn how to navigate it.

PARTNERS

Lift off and aiming high for 2018

Written by Declan Noone, EHC PARTNERS programme consultant

November 28th saw the official launch of the EHC Procurement of Affordable Replacement Therapies - Network of European Relevant Stakeholders, or PARTNERS, programme in the EU Parliament hosted and attended by Members of the European Parliament (MEPs) Norica Nicolai (Romania), Cristian Busoi (Romania), Nessa Childers (Ireland) and Wim van de Camp (Netherlands). Camille Bullot from the European Patients' Forum (EPF) and Yann Le Cam from EURORDIS – Rare Diseases Europe also spoke



MEP Norica Nicolai chaired the launch event

at the launch, highlighting the innovative approach of the programme and the hope that this could provide access in a number of European countries with current limited access to Factor VIII and IX for haemophilia but also a potential trail blazer for other conditions in trying to improve care for patients. Since our last article (see August newsletter), Albania, Latvia, Macedonia, Azerbaijan, Romania and Bulgaria have received the initial assessment visit to assess the current situation in terms of haemophilia care and determine the best approach for the implementation of the PARTNERS programme. There have been multiple meetings with regulatory authorities, government ministers, hospital management and insurance agencies. From these meetings, the overarching principles remain steadfast in terms of requiring national co-ordination in relation to a centralised procurement process, which involves patient representatives and clinicians as well as committing to

a three-year contract to increase supply towards double the current use of 4 International Units (IU) per capita of Factor VIII and 0.5 IU for Factor IX. However, it is clear a “one-size fits all” approach will not be possible. This is apparent in terms of national legislation, current and future procurement agencies and changes in procurement practises. So far, all stakeholders have very warmly received the programme and there is a clear willingness to engage in PARTNERS in order to raise the standard of care towards the Council of Europe recommendations for haemophilia care in the eligible countries.

As this is the December edition of the EHC newsletter and hence a time for reflection on the year passed and thoughts for the one to come, I will borrow a phrase Londoners use to sum up what needs to be done in the coming months. “Please Mind the Gap.” My - and indeed our - hope for the PARTNERS programme reaches far beyond a procurement programme. This is a significant shift in the way medications are purchased in many countries moving beyond the six-month or annual procurement contracts that are currently in place. In terms of simply procurement, this has led to gaps in supply while additional contracts are still negotiated and often supply is further delayed until the next budget cycle. In terms of patients, this



Speakers from the night included Zafar Yuldashev, UNDP (2nd from left), Yann Le Cam, CEO of EURORDIS, and Camille Bullot, Director of Operations and Engagement at EPF

has meant that a parent must stand outside of a clinician's office, waiting whatever length of time she or he has to, to beg for medication, which is not available because there has been a postponement of the tender. This has also meant that a patient going to the hospital with a headache and slipping into a coma has still gotten no treatment because the bleed happened outside of a current procurement cycle. These are the stories I have found on the ground and this is why this PARTNERS programme is not, and should never be, about the price of an IU!! It is about working with all stakeholders to agree on the continuity and predictability of supply within a health care system to benefit patients, clinicians and payers in improving the standard of care for haemophilia.

The EHC are very aware of the gap that is currently present between countries in terms of the provision of haemophilia care and the aspects that need to be improved. We are also aware of the significant impact of new treatment availability, which has the potential to widen that gap at a faster pace than ever before. So for 2018, let us "Please Mind the Gap" and work together to make the New Year an evolution in terms of procurement, supply and availability of haemophilia treatment products led by the PARTNERS programme.

On the next page, you will find the press release on Kedrion Biopharma and Swedish Orphan Biovitrum (Sobi) signing a memorandum of understanding (MoU) with the EHC in support of PARTNERS.

Follow us on Facebook and Twitter for regular updates on the programme.

Brussels – December 22nd 2017

Kedrion Biopharma and Sobi sign on to ground-breaking PARTNERS programme working towards sustainable haemophilia treatment and care in Europe

Kedrion Biopharma, an Italian plasma-derived haemophilia treatment manufacturer, and Swedish Orphan Biovitrum AB (Sobi™), supplier of extended half-life (EHL) haemophilia treatment products, signed a Memorandum of Understanding (MoU) with the European Haemophilia Consortium (EHC) in support of the EHC **Procurement of Affordable Replacement Therapies – Network of European Relevant Stakeholders (PARTNERS)** programme. Through this agreement, the companies commit to supporting the PARTNERS programme to increase access to vital treatment for over 5000 people with haemophilia in European countries that currently significantly undertreat haemophilia patients, substantially improving their quality of life.

Background

Through systematic data gathering and close collaboration with its National Member Organisations (NMOs), the EHC has long been monitoring the level of haemophilia care throughout Europe. Though there have been improvements in many areas, there remain disparities in access to treatment and quality of care for people with haemophilia. In several countries with developed health care systems but limited budgets, sustainable levels of factor replacement to effectively treat haemophilia are not available. This results in lack of treatment or under-treatment, which in turn can lead to debilitating, or even potentially fatal, consequences for the people with haemophilia in those countries. It also places a burden on those countries' broader health and social systems.

PARTNERS programme

To address these urgent issues, the EHC has developed a new and innovative approach to create a sustainable procurement model for treatment for haemophilia A and haemophilia B in countries located both inside and outside of the European Union. The main objectives of the PARTNERS programme are to work with national health care systems in order to:

1. Enable countries to provide adequate levels of factor concentrate that will result in improved levels of treatment for people with haemophilia. Governments will be able to purchase much larger quantities of quality treatment without significantly increasing their national haemophilia budget.

2. Formally involve haemophilia clinicians and patient representatives in the national procurement process in an effort to inform selection and the sustainability of health



care systems. In the experience of the EHC, this type of model leads to a more cost-effective process.

3. Encourage collaboration between all stakeholders, including companies willing to participate in a fair and transparent tendering system that seeks to improve standards of care for people with haemophilia within these countries.

The criteria for country participation in PARTNERS are based on the latest set of recommendations from the Council of Europe's European Directorate for the Quality of Medicines and Healthcare (EDQM) consensus meetings for the optimal treatment of haemophilia in Europe. Eligible countries are those that **do not** meet the recommended minimum treatment levels of 4 International Units (IU) factor VIII replacement therapy per capita (2016 recommendations), minimum treatment levels of 0.5 IU factor IX replacement therapy per capita (2016 recommendations) and **do not** provide prophylactic treatment for all children with severe haemophilia. These minimum levels of treatment are recommended on the basis that they allow for all children to be on some form of prophylaxis, all adults to be at least on on-demand treatment with home therapy, no restriction on a required surgery and possible access to Immune Tolerance Therapy for patients with inhibitors. Governments need to also agree to formally include haemophilia clinicians and patient representatives in national tender/procurement systems as part of the supplier bid evaluation process (2016 recommendations).

Brian O'Mahony, EHC president:

"Today in Europe we still see countries where people affected by haemophilia have the same level of joint damage and poor quality of life as we saw in patients before the advent of modern coagulation factor concentrates in the 1970s and this is unacceptable.

We believe that, with this programme, existing national haemophilia budgets can lead to increased quantities of treatment purchased through better tender systems. This ambitious project aims to provide an increased access to treatment but also to include patients and health care professionals in the decision-making process of the organisation of haemophilia care in any given countries. We are also very thankful for the support of all of our partners without which this project would not become a reality."

Stakeholders

One of the most important aspects, without which this programme would not be possible, is the involvement of pharmaceutical companies. Companies supplying treatment products for haemophilia A and haemophilia B (namely Factor VIII and FIX replacement therapies, respectively) that have signed a Memorandum of Understanding with the EHC and who support the PARTNERS programme, express willingness to participate in national tender processes of the eligible and participating countries under standard, bi-lateral and confidential bidding conditions within the national procurement processes in a way that enables the increase in purchasing commitment from the national government in an affordable way.

Lorenzo Melani, Hemophilia Marketing Manager, Kedrion Biopharma:

"Nowadays, we know very well how a patient needs to be treated. Now that we know the goal, we have to speed up the process. We strongly believe that a breakthrough programme, such as PARTNERS, will help patients in a number of countries where treatment levels are not optimal."

Philip Wood, Head of Haemophilia, Sobi:

The PARTNERS programme has the potential to create one unified voice amongst physicians and patients in the tender process. This approach takes sustainable access to a whole new level as people living with haemophilia will have the possibility to access the latest treatments on a long-term basis, raising the standard of care.

Current standing

PARTNERS has received endorsement from the European Patients' Forum and EURORDIS – Rare Diseases Europe, as well as from many Members of the European Parliament (MEPs). To solidify this support on both a European and national level, the EHC officially launched the PARTNERS programme on November 28th in the European Parliament in the presence of MEPs, EU institutions, pharmaceutical companies and patient organisations involved in PARTNERS.

In 2017, the EHC visited several of the eligible 14 countries for a meeting with representatives of their Ministries of Health, Insurance Funds and haemophilia patient organisations to discuss interest in the programme, and an implementation schedule is now being elaborated on.

About Haemophilia

Haemophilia is a rare congenital bleeding disorder resulting from a deficiency in either clotting factor VIII (haemophilia A) or clotting factor IX (haemophilia B), which are essential proteins for the blood clotting process.

Having haemophilia means that when a blood vessel is damaged in that individual's body, the bleeding that occurs lasts for a prolonged amount of time. These bleeds can either be spontaneous or they can be caused by trauma and stress. If the bleed is not stopped with treatment products and properly managed, it can severely and permanently damage the patient's joints (such as ankles, hips and knees), causing affected individuals to have significantly reduced mobility, increased pain and decreased quality of life. Untreated bleeds can also lead to death.

About Kedrion Biopharma

Kedrion Biopharma is an international company that collects and fractionates blood plasma to produce and distribute plasma-derived therapeutic products for use in treating and preventing serious diseases, disorders and conditions such as haemophilia, primary immune system deficiencies and Rh-sensitization.

Kedrion acts as a bridge between donors and the people who need treatments, and works on a global scale to expand patients' access to available treatments.

Additional information about Kedrion Biopharma can be found at www.kedrion.com.

About Sobi

Sobi is an international specialty healthcare company dedicated to rare diseases. Sobi's mission is to develop and deliver innovative therapies and services to improve the lives of patients. The product portfolio is primarily focused on Haemophilia, Inflammation and Genetic diseases. Sobi also markets a portfolio of specialty and rare disease products across Europe, the Middle East, North Africa and Russia for partner companies. Sobi is a pioneer in biotechnology with world-class capabilities in protein biochemistry and biologics manufacturing. In 2016, Sobi had total revenues of SEK 5.2 billion (USD 608 M) and about 760 employees. The share (STO: SOBI) is listed on Nasdaq Stockholm. More information is available at www.sobi.com.

Additional Resources

For a detailed description of the consensus recommendations coming out of the EDQM meetings, also known as the "Wildbad Kreuth" meetings, please visit:

<http://www.ehc.eu/kreuth-iv-consensus-recommendations/>

For more information on the PARTNERS programme, please visit:

<https://www.ehc.eu/partners/>

To learn more about haemophilia and other congenital bleeding disorders, visit:

www.EHC.eu

Haemophilia Stories is a documentary produced for the EHC, which depicts the daily lives of people with haemophilia in five European countries. It shows the different outcomes that disparities in access to treatment lead to:

<https://www.ehc.eu/document/haemophilia-stories-short-videos/>

Inhibitor Stories is a follow-up documentary that focuses on people with haemophilia and inhibitors and the various challenges they face:

<https://www.ehc.eu/community/2017/10/30/inhibitor-stories-documentary-living-haemophilia-inhibitors/>

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Meet the EHC Medical and Scientific Advisory Group

Close collaboration with a comprehensive team of medical experts is essential to the work of the European Haemophilia Consortium (EHC). In addition to our already established committees, earlier this year, the Medical and Scientific Advisory Group (MASAG) was formed with the objective to:

- Help ensure a multidisciplinary approach to the work of the EHC,
- Provide advice and guidance on specific areas of EHC engagement,
- Provide strategic input into specific event programmes and activities as necessary, and
- Provide hands-on, in person support in key events as needed.

Many of you are probably already well-acquainted with their work but here, we would like to introduce you all 16 members of MASAG and the areas of expertise that they bring into the group.

We are grateful for their enthusiasm in joining the MASAG and for their contribution to our continued efforts to improve treatment and care for all people with haemophilia, von Willebrand Disease and other rare bleeding disorders in Europe!

**Massimo Colombo –
Hepatologist**



Massimo Colombo is Director of the Center for Translational Research in Liver Disease at the Humanitas Research Hospital, Rozzano, Italy. In the past 15 years he has been Chairman of the Division of Gastroenterology and Hepatology and Chairman of the Department of Medicine and Liver, Kidney, Lung, Bone Marrow and Organ Transplantation at IRCCS Maggiore Hospital in Milan, Italy. He has also been a Professor of Medicine and Gastroenterology at the University of Milan for more than 25 years. In September 2016 he was appointed Chairman of the European Association for the Study of the Liver (EASL) International Liver Foundation.

Alison Dougall – Dentist



Alison Dougall is a clinical consultant in special care dentistry at the Dublin Dental University Hospital (DDUH). She is also involved in the teaching and training of dentists at Dublin University Hospital at undergraduate, postgraduate and doctorate level. She is the chair of the Clinical Audit Committee. As part of her role at DDUH she provides consultant-led dental clinics within St Vincent's University Hospital and St James's Hospital in Dublin, Ireland. In 2014, Dr Dougall was elected as Chair of the Dental Committee of the World Federation of Hemophilia (WFH).

Carmen Escriola-Ettinghausen – Paediatrician



Carmen Escriola-Ettinghausen is the Director of the Haemophilia Centre Rhein Main - HZRM in Frankfurt, Germany. She has held this position since 2015. She is a member of the German, Swiss and Austrian Society for Thrombosis and Haemostasis Research (GTH) and the International Society for Thrombosis and Haemostasis (ISTH).

Dan Hart – Immunologist



Dan Hart is a senior lecturer in haematology and immunobiology at Barts and The London School of Medicine, Queen Mary University London and Honorary Consultant Haematologist at The Royal London Hospital Haemophilia Centre, Barts Health NHS Trust. He is part of a multidisciplinary haemophilia team at one of the country's largest haemophilia centres and has an evolving research programme at Barts and The London School of Medicine and Dentistry exploring immunological complications of haemophilia treatment using computational biology, proteomics and next generation sequencing strategies.

Anneliese Hilger – Regulatory Advisor



Anneliese Hilger is currently working for the Paul-Ehrlich-Institut in Germany where she is the head of Section "Blood Coagulation Products." She is responsible for plasma-derived blood products or biotechnological analogues dealing with regulatory, non-clinical and clinical aspects in charge of marketing authorisation (national, Mutual Recognition Procedures, Decentralised Procedure, scientific evaluation of clinic trial applications and national and European scientific advices.)

Vince Jenkins – Geneticist



Vince Jenkins has over 25 years' experience in the field of haemostasis and haemophilia. He was trained and worked at the Katharine Dormandy Haemophilia Centre, Royal Free Hospital, becoming Chief Medical Scientist.

In 2004, he moved to the National Centre for Hereditary Coagulation Disorders, Dublin, Ireland. Here he set up and developed a new laboratory for the molecular diagnosis of haemostatic disorders and continued research in haemophilia and von Willebrand Disease.

**Rezan A Kadir –
Gynaecologist**



Professor Rezan A Kadir is a Consultant Obstetrician and Gynaecologist with a sub-specialty in foetal medicine at the Royal Free Hospital in London, United Kingdom (UK). She is also a Specialist Consultant in women with bleeding disorders at the hospital, and an Honorary Reader with the University College School of Medicine. She has set up a multi-disciplinary clinic in the haemophilia centre at the Royal Free Hospital in London, which is jointly run with a haemophilia specialist, nurse and family counsellor and therapists. This clinic has been unique in providing comprehensive care for women in families with bleeding disorders.

**Piet de Klein –
physiotherapist**



Piet de Klein graduated as a physical therapist in 1979 in Utrecht, the Netherlands and has since been involved in haemophilia care. Since 1988, he has been fully responsible for the physiotherapy of visiting haemophilia patients at the Van Creveld kliniek located in the University Medical Centre of Utrecht. He has also been a member of the World Federation of Hemophilia (WFH) Musculo-Skeletal Committee (MSK). Mr de Klein is also a board member of the Physiotherapy Working Group of the European Association for Haemophilia and Allied Disorders (EAHAD).

"Comprehensive care starts from a young age and should really be comprehensive. Functional recovery after each bleed is key. Only together we can do it!"

Piet de Klein

**Alec Miners – Health
Economist**



Alec Miners is an Associate Professor of Health Economics at the London School of Hygiene and Tropical Medicine. He has a particular background and interest in undertaking and using cost-effectiveness analysis. He also has a long-standing interest in haemophilia – his PhD from the University College London (UCL) was on ‘The economics of replacement therapy for people with severe clotting factor disorders.’

**Beatrice Nolan –
Paediatrician**



In 2008 Beatrice Nolan was appointed full-time Lead Consultant Haematologist in relation to paediatric haemophilia and allied bleeding disorders at Our Lady's Children's Hospital in Crumlin (OLCHC – Dublin, Ireland) with a national remit. Previously she worked as a Consultant Haematologist at the St James's Hospital in Dublin, Ireland.

Beatrice is a member of the National Haemophilia Council (NHC) of Ireland and the Irish Haemophilia Product Selection Monitoring Advisory Board (HPSMAB).

"I agreed to join the MASAG group because I've always thought that the work of the EHC in trying to address discrepancies across Europe in haemophilia care is important."

Beatrice Nolan

[Not Pictured] Cristina Novembrino – Laboratory Scientist

Cristina Novembrino is a researcher focusing on diagnostic activity and management of research protocols in thrombosis and haemostasis at the Fondazione IRCCS Ca' Granda – Ospedale Maggiore Policlinico, in Milan, Italy. Since 2010, she has held various research positions in this institution. She is currently working in the team of Prof Flora Peyvandi and Dr Ferruccio Ceriotti.

[Not Pictured] Roseline d'Oiron – Women and Bleeding Disorders Specialist

Roseline d'Oiron is currently a clinician investigator at the Reference Centre for Haemophilia and Other Congenital Rare Bleeding Disorders, Congenital Platelets Disorders and von Willebrand Disease at Bicêtre Hospital AP-HP – Paris XI University, Le Kremlin-Bicêtre, France.

Her research interests are principally in the area of haemophilia (especially molecular mechanisms, inhibitors, immune tolerance, mild/moderate haemophilia and carrier issues) and Glanzmann's thrombasthenia. She has been a member of the Executive Committee of the European Association for Haemophilia and Allied Disorders (EAHAD).

**Jamie O'Hara – Health
Economist**



Jamie O'Hara is an industry economist by background with expertise in practical and technical applications of the discipline across industries. He is currently the health economic lead on an expert panel piloting proof of concept for the development and implementation of a global haemophilia registry. He is also a Senior Lecturer in Health Economics at the University of Chester.

Debra Pollard – Nurse

Debra Pollard is the Lead Nurse at the Katharine Dormandy Haemophilia & Thrombosis Centre at the Royal Free Hospital in London. She has extensive experience in the care of both adults and children with inherited and acquired bleeding disorders. She is a member of the Clinical Advisory Group for the UK Haemophilia Society and is also on the editorial board of the *Journal of Haemophilia Practice*. She is a Trustee of Haemnet, a charity, which supports health and social care professionals promoting excellent care for people with bleeding disorders.

Frits Rosendaal – Epidemiologist

Frits Rosendaal M.D. Ph.D., is professor of clinical epidemiology at the Leiden University Medical Centre, the Netherlands.

In the field of thrombosis, he designed the Leiden Thrombophilia Study (LETS) and the Multiple Environmental and Genetic Assessment of risk factors for venous thrombosis (MEGA), that have been instrumental in the identification of a series of risk factors for thrombosis. He has been Chairman of the Council of the International Society for Thrombosis and Haemostasis (ISTH).

[Not Pictured] Glenda Silvester – Regulatory Advisor

Glenda Silvester worked for 18 years at the European Medicines Agency (EMA), where she was the lead for blood products. She was Scientific Secretary of the Blood Products Working Party, which provides recommendations on matters relating to the efficacy and safety of blood products.

She retired from her post of Principal Scientist, Quality of Medicines in July 2015. Prior to her work at the EMA, she also worked for the UK Medicines Agency and in the pharmaceutical industry. She is also a pharmacist.

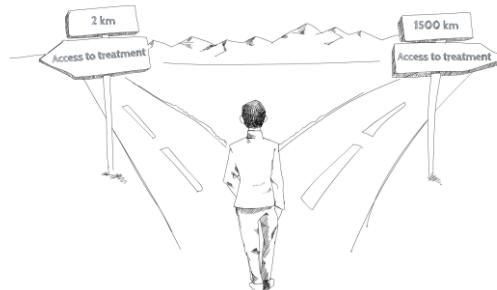
"I hope that my experience in the regulation of medicines for patients with haemophilia, gained in the 18 years that I worked at the EMA, may be helpful to the EHC."

Glenda Silvester

Recommendations on the Optimal Treatment of Haemophilia in Europe

Written by Raia Mihaylova, EHC Communications Officer

2017 is ending with an important victory for the haemophilia community! In mid-December, the Council of Europe Committee of Ministers announced that they are updating their recommendations on haemophilia treatment and care in Europe to include the 2016 European Directorate for the Quality of Medicines and Healthcare (EDQM) recommendations. This set of recommendations includes the prioritisation of hepatitis C treatment for people with haemophilia, access to immune tolerance induction (ITI) for all people with inhibitors, and for the **minimum** consumption of FVIII to be 4 International Units (IU) per capita of the general population and 0.5 IU/capita for FIX in every country (full list of the 2016 recommendations can be found below).



This update brings together the EDQM recommendations from 2013, previously endorsed by the Council (can be viewed [here](#)), with the 2016 EDQM recommendations. Together, they serve as a powerful tool to advocate for improvements in haemophilia care and access to treatment on a national level, while their endorsement by the Council aids in calling for their implementation within each country. To highlight their significance, the EHC is currently working on an advocacy video that emphasizes the need for the recommendations – look out for it on our social media pages in the upcoming days.

Lets make 2018 the year all people with haemophilia in Europe receive quality care and full access to treatment!

EDQM 2016 Recommendations

1. Hospitals providing clinical care for people with haemophilia and related disorders are strongly recommended to seek formal designation as either EHCCC or EHTC. (Access to comprehensive care and replacement therapy should be equitable in all parts of a country).
2. There should be agreed national protocols or guidelines on management of the ageing patient with haemophilia. Treatment centres are encouraged to include an appropriate general physician in the comprehensive care team.
3. The minimum consumption of factor VIII concentrate in any country should be 4 ~~iu~~/capita of general population. (Data expressed as units/severe patient should also be collected in parallel in future.)
4. The minimum consumption of FIX concentrate in a country should be 0.5 ~~iu~~/ per capita of general population.
5. Treatment for hepatitis C with direct-acting antiviral agents should be provided to all people with haemophilia on a high priority basis.
6. Genotype analysis must be offered to all patients with severe haemophilia and the results used to identify carriers in the wider family.
7. People with inhibitors should have access to immune tolerance.
8. People with inhibitors should also have access to elective surgery at a specialist centre with relevant experience.
9. National or regional tenders for factor concentrates are encouraged and should always include both haemophilia clinicians and national haemophilia patient representatives.
10. Outcome data including health related quality of life should be collected with appropriate study design, e.g. annualised bleed rates (ABR), mortality, joint score and time off education or employment.
11. Treatment with extended half-life factors should be individualized and protection against bleeding should be improved by increasing trough levels.
12. There is increasing evidence that the incidence of inhibitors amongst previously-untreated patients (PUPs) varies between products. Steps should be taken to understand and minimise this risk. (Patients, or their parents, should be involved in discussions related to product choice.)

NMO NEWS

Meet Marko Marinic, the new president of the Croatian NMO

*Interview with Marko Marinic, President of the Croatian Haemophilia Society, EHC Croatian NMO;
Interview taken by Raia Mihaylova, EHC Communications Officer*



Can you briefly introduce yourself and your involvement with the Croatian Haemophilia Society until now?

A little about me – I have a PhD in the field of bioethics. My doctoral thesis was about the value of human life in a contemporary society through the perspective of a person with a disability, which I just published a book on. I currently work at the Institute of Social Sciences “Ivo Pilar” in Zagreb as a senior research associate and I am also a professor at the University of Zagreb, where I teach bioethics and measurements of the quality of life of people with disabilities. The prime area of my research at the institute is exactly that: the quality of life of people with disabilities and people with chronic conditions.

At the institute, the primary work is empirical research among the Croatian population. My specific area is, as I mentioned before, people with disabilities and chronic diseases. We do many surveys; one such was done five years ago on the quality of life of adults with haemophilia in Croatia. We received a lot of responses and we presented the results at different conferences, such as the Congress of the World Federation of Hemophilia and other scientific conferences, as well as publishing a scientific paper based on that research.

In the survey, we asked many different questions related to quality of life and looked at various variables – self-esteem, happiness, social activities, intimate life, daily life, etc.

The results showed that there are a lot of problems, especially for the older population with their joints and atrophy. One interesting conclusion we had is that people with haemophilia must have a higher education. There is a huge decrease in happiness and overall quality of life that happens when people with haemophilia reach their 40s. They have problems with walking, with working, particularly any job that includes physical activity – they reach a point where disability doesn't allow them to perform well anymore and they lose their job. Education is really important so that people can access positions that

don't require physical labour. This is the reason why this year, at the Society, we are starting a student grants programme for children with haemophilia. We will try to encourage them to aim for a higher level of education. Young people with haemophilia and other rare bleeding disorders, who are either in secondary school or in university and who are members of our Society can apply for this grant. It will be for every school year, every month they will receive a set amount. We will put up this call for applications in the beginning of next year. This is something I really wanted to focus on when I became president.

For my involvement with the Croatian Haemophilia Society, I have been a member since the beginning of the Society in the 1990s. For the last seven years, I have been chief editor of our journal *Haemophilia*, which comes out once a year.

What is your vision for the future of the organisation?

We can state the primary goal in one sentence: to achieve better quality of life for people with haemophilia in Croatia, not only in a medical treatment sense but in every other sense as well. Of course, the medical questions are the most important and we will do everything that we can do to get the best treatment for our community.

What are some challenges you are facing at the Society?

The availability of factor concentrates in Croatia is okay but it should be better. We will try to increase the level of International Units (IUs) per capita and we will try to follow the new treatments and provide access to them, as soon as possible. We want to have the same treatment as is available in the best centres in the world, and we will do our best to achieve this. There are huge improvements in the field of haemophilia nowadays and there are many new treatments on the horizon. Also, we would like to improve the overall comprehensive care of haemophilia – orthopaedic services, dental work, psychology, physiotherapy – we have many initiatives in this regard. We have a good level of care but we will try to make it even better.

Another goal is to improve access to new treatments for the comorbidities people with haemophilia have, such as Hepatitis C (HCV). There are some people who are already cured but we, of course, want everyone to be cured. We think that this will happen really soon. There were a lot of problems a couple of years ago in providing access to the new direct-acting antiviral treatment for HCV, but now it is much better. We have an agreement with the government that every single person with haemophilia who was infected with HCV will receive the treatment.

What are some of the current initiatives/activities that you have at the Croatian Haemophilia Society?

There is our summer camp, this year it was the 11th edition. Every year, almost 200 participants come from all over Croatia. At every single camp we have many presentations from medical experts, workshops on physiotherapy and psychology, and other things that are important for people with haemophilia. We also invite and get together with government representatives, people from the Ministry of Health, health insurance, physicians, pharmacists, and of course, people with haemophilia and parents of children with haemophilia. When we are together, it is easier to solve a problem. Many of our problems are solved exactly at these camps.

Some other activities: we always mark World Haemophilia Day in a couple of locations throughout Croatia. We also organise many additional workshops throughout the year at our hospital, for example, for people who are new to the whole experience with haemophilia, or for people who don't adhere

well to their medication and how they can improve that. And then there are all the social activities we do, they are always good for sharing experiences.

So there is quite a big focus on young people?

Yes. There is the problem of not having many younger people who are really involved in the work of the Society. I know this is a problem for many of the Societies as the younger generation has good treatment and they don't feel the need to be so actively involved. In a way it is a positive problem but we will try to involve them as much as we can. Besides the student grants that I mentioned, we will organise some clubs for them where they can communicate their wishes for things they would like to see happen at the Society, and we will work to meet these goals.

What is something that will surprise people about you?

I don't know something that will necessarily surprise them. I am a regular haemophilia guy and that is enough to be special! 😊

New President of the Czech NMO

Interview with Martin Bohún, President of the Czech Haemophilia Society, EHC Czech NMO; Interview taken by Raia Mihaylova, EHC Communications Officer

Can you briefly introduce yourself and your involvement with the Czech Haemophilia Society up until now?

Since I was seven years old, I attended the annual haemophilia summer camps of the Society. This went on until I was 18. Afterwards, I started to organise the camps through my role as an instructor, until I became the main coordinator of the summer camps. I have been a member of the Czech Haemophilia Society (CHS) Steering Committee since 2002. I also actively participate in the organisation of various other events held by the Society, such as scientific conferences, workshops, etc.

On a more personal level – I was born in 1981. I studied at and graduated from business school and nowadays, I work in IT support. Despite some health limitations, I do my best to live an active life – I swim, I ride a bike and a snow bike, and I like to explore new places. I like to constantly learn new things.

What does being president of the Society mean to you? How do you see this role?

In the first place, it means responsibility, but it's a pleasure as well. You know, all my best friends are somehow connected to haemophilia, which means I can talk with them about my everyday joys and sorrows. After the death of my brother, also a haemophiliac, I decided that the situation for people with haemophilia in our country isn't something I can be indifferent to. That is when I made the decision to join the CHS Steering Committee, whose president in those times was František Vondryska – I'm sure many of you remember him. Gradually, I got to know the activities, I became the main coordinator of the summer camps, I integrated into the haemophilia community in the Czech Republic. Everyone around me helped me to realise more and more why I was there and what more needed and could be done. I definitely wouldn't have been able to do anything without the great support from my friends, my family and doctors.

How do you see the future of the Czech Haemophilia Society - what are some things you would like to accomplish?

We discuss this topic all the time. The Society should be, above all, a platform for people with the same troubles. I would like the Society to be a trusted institution, which our members can rely on and which can be contacted upon any difficulties. Many impulses for our work result from the yearly cross-generation meeting of haemophiliacs that we hold. Although it might seem that there is nothing new to offer, the truth is different. The experts attending the meeting come up with new and interesting topics every year, and these topics are further discussed by participants during individual or group workshop sessions. This seminar provides us with new insights and also makes it possible for everyone to meet new friends. Some haemophiliacs attend to gain some experience concerning total endoprosthesis, others discuss pharmacokinetics, vein care and so on. Participants are also interested in gene therapy, timing of FVIII/FIX infusions, medications connected with cardiovascular issues, vaccination, extended half-life factors or issues regarding female carriers of haemophilia. One new activity is a workshop for teenagers. In a friendly environment, the young generation can talk with and share their concerns with the older patients, so that they can fully realise how treatment has developed over time. To sum up, there is no reason to say „I already know everything, I don't get anything new from such events.” And even if someone has a lot of knowledge and experience, they

can still help someone who visits such an event for the first time and seeks help or advice. Knowledge of the latest trends and emerging treatments is crucial.

In the future, we are aiming to develop activities for patients with von Willebrand disease as well and to integrate them better into the active life of the Society.

You largely touched upon it in the previous question, but are there any other initiatives of the CHS, that you would like to mention?

The CHS provides support to people with haemophilia in negotiations with health insurance companies, doctors and local authorities. The Society also keeps its members, as well as medical workers, informed and contributes to the dissemination of new treatment information. In order for every member to know as much as possible about their condition, we aim to play an important role in their and their relatives' education. Patients can meet each other at regional meetings, improve their physical condition during the adults' camps and there are also special summer camps for children. Twice a year, we organise one-week stays for people with haemophilia and inhibitors at a spa, we meet at interesting and adventurous places to mark World Haemophilia Day, we contribute to the education of physiotherapists in haemophilia centers, and so on.

There is a survey called „The Life of Czech Haemophiliacs, “ which I consider to be unique. The first edition took place in 2011, followed by a second one at the end of 2016. The vast majority of questions were present in both versions of the survey, so that it is possible to analyse the change in situation and attitudes. However, there were some new questions added to the second version, as some new trends emerged – factors with extended half-life, gene therapy or pharmacokinetics. All of these are signs of positive development, which was definitely captured by the survey.

What is one thing that would surprise people about you?

Probably, this is a question for others ;-) Life itself brings many surprises. Sometimes the situation is joyful, sometimes painful and affects our emotions. Still, I strive to behave kindly, humbly and be persevering.

I wholeheartedly thank my colleagues from the previous Steering Committee of the Czech Society of Haemophilia. I very much appreciate the cooperation with the previous presidents of the Society Mr Michal Stastny, Mr Frantisek Vondryska and Mr Vladimir Dolejs. Thank you for everything you did for haemophiliacs in our country.

Activity Grant Report: International basketball camp for people with haemophilia 2017

By Egidijus Sliauzys, President of the Lithuanian Haemophilia Society, EHC Lithuanian NMO

The new basketball season in Lithuania started in October. The Lithuanian Haemophilia Society basketball team, BC "LZSHA", again competed in the KKML, the Kaunas basketball league. For the Lithuanian blood brothers, this was nothing new. Haemophilia is a serious medical condition and various activities could lead to having a bleed. But with the available treatment in Lithuania, swimming, cycling, diving or even playing basketball is something natural for the country's haemophilia population. However, many people with haemophilia in Ukraine, Belarus and other countries think quite differently about the possibility of running in a basketball court. Without sufficient amount of therapeutic products even for on-demand treatment, let alone for prophylaxis, sports and haemophilia seemed to be incompatible.



The annual basketball camp of the Lithuanian Haemophilia Society is a unique place for local and foreign young men between the ages of 15-35 to enjoy the freedom of movement and to learn some basketball tricks. This year in the last week of July, men with haemophilia from Poland, Ukraine, Belarus, Armenia, Slovenia and Bulgaria came to the haemophilia rehabilitation center „Tulpé“ in Birštonas. Although the treatment

options for haemophilia in the neighbouring countries and in further Slovenia are similar to those in our country, guys from these countries were excited to join us for an active week of sports. They were attracted by the opportunity to do sports together with other haemophiliacs, by the attention of professional coaches and physicians and the basketball country itself.

The camp was also attended by the long-time "LZSHA" team players and rookies from Lithuania. The final result of the training week was a friendly match between the Lithuanian and foreign guest teams. This year "LZSHA" won against the "H force," with the final result 66:49. A week is quite a short time to learn the essentials of basketball, but professional coaches Indrė Keršulytė, Justas Ališauskas and Eglė Žibinskaitė did not let the guys rest in the sports hall. Many of the participants needed intensive strengthening exercises, which were supervised by physiotherapist Greta Baronaitė in the swimming pool and at the gym. For most, the camp was an experience that they cannot wait to return to. Lukas Mazurkiewicz, a great basketball admirer who participated in the camp for the second time, said that this



project helped to fulfill his dream - to play in a basketball match despite his health condition. Slovenians Renato and Miha had a great time and not only did they try to learn Lithuanian words, they also did not spare praise for this unique project, calling attention to the lack of similar events in their own country.

The project was also presented at the European Haemophilia Consortium (EHC) 2017 Conference in Vilnius, drawing attention and interest from an even broader public. We hope that during the next summer of 2018, the orange balls will again soar through the basket, thrown by an even larger group of blood brothers.

The EHC made participation in this camp by people with haemophilia from neighbouring countries possible through an EHC Activity Grant.

Analysis of medical and social surveys of patients with haemophilia; setting the strategy for the next five years

By Gulnara Huseynova, President of the Azerbaijan Patients with Haemophilia Society, EHC Azeri NMO

On November 17th, the Azerbaijan Patients with Haemophilia Society presented the final results and report of a survey conducted among haemophilia patients to understand their quality of life. The survey was carried out within the framework of a project called "Evaluation of the State Program on Haemophilia," with financial support from the Council on State Support to Non-Governmental Organisations under the auspices of the President of the Republic of Azerbaijan.



More than 225 people with haemophilia took advantage of the project, which along with the survey, included the option of undergoing medical examinations and an analysis of health status. The comprehensive examination was composed of laboratory analysis (if necessary), orthopaedic and dental examination, as well as ultrasound and X-ray imaging, which were offered to patients with haemophilia between the ages of 12-35.

All the results have now been analyzed and a package of proposals of key improvement areas will be prepared and submitted to the Ministry of Health and the Ministry of Labour and Social Protection. They will also receive a report on the degree of disability of patients with haemophilia; those citizens who are in urgent need will be offered rehabilitation, with the assistance of our organisation.

The analysis of the medical results and survey showed that:

- Among the 225 patients studied, five of them are disabled in a 1st category, 37 are disabled in 2nd category, 107 patients in a 3rd and 76 patients have no disability.
- Because of the absence of a genetic laboratory in Azerbaijan, probability was used to count the approximate number of carriers of the haemophilia gene. The calculated number was 1,500.
- 109 patients above the age of 18 had serious dental problems.
- Of the 109 patients older than 25 years of age, 37 patients suffered cranio-cerebral haemorrhages of varying degrees.

Recommendations made based on the orthopaedic examinations included:

- Orthoscopic surgery for 17 patients older than 25 years,
- Knee joint endoprosthesis for seven patients older than 35 years,
- Orthopaedic surgery for three patients older than 35 years,
- Reduction of body weight for 18 patients over 25 years,
- Intra-articular injections for 67 patients older than 25 years,
- All 225 patients were recommended for physiotherapy and rehabilitation,
- Therapeutic gymnastics and walking was recommended to patients over 12 years of age, in

cases where no other serious complications were present.

The Azerbaijan Patients with Haemophilia Society will adapt its strategic plan for the next five years based on these recommendations and findings so that we can concentrate our efforts on where they are most needed.

IN LOVING MEMORY

Vladimir Ilijin (1956-2017), President of the Serbian Haemophilia Society, a great man and friend



Vladimir was actively involved in the work of the Serbian Haemophilia Society (SHS) since its establishment in 2000 and for the past eight years, he was the Chairman of the Steering Board. As a person with severe haemophilia A with inhibitors, he had fragile health, but also a very strong will. He selflessly fought for the brighter future of people with haemophilia and sought to secure patients in Serbia better treatment.

It was never difficult for him to do anything for the Society, nor for any of us. We always received true and honest advice, whether they were problems we were coping with collectively for a long time, or dealing with personal problems. He was a voice of reason and a true friend. He enriched us with all his goodness and wise words. He was respected and esteemed far beyond the framework of the Society, by the health care workers with whom we cooperated, representatives of state institutions and other associates, as well as by our friends outside Serbia.

A great strategist with a vision of how to fight, he planned and measuredly solved one problem at a time. New drugs, home therapy, prophylaxis for children and adults, and increasing the amount of treatment are just some of the achievements we won under his guidance. He represented the Society on the Tender Committee at the National Health Insurance Fund, as well as at the National Haemophilia Committee. Side by side, he was sitting with economists and the best haematologists in our country, advocating for all patients in Serbia whether they were from a small village in the south, or from a large city in the north. He always said that we are all equal and that all patients in our country have the right to equal treatment.

He advocated for active SHS cooperation with the World Federation of Haemophilia (WFH) and the EHC and supported all common activities. This contributed to the realization of over 15 workshops on topics such as dental, musculoskeletal, psychological health and many others, both for members of the Society and for medical workers. He pledged that we can raise the level of treatment through knowledge.

Always open to new ideas, he supported the establishment of the Youth and Parenting Group. He leaves an immeasurable contribution to the work of the Society, as well as to improving the quality of treatment for people with haemophilia in Serbia. He devoted years of his life to his vision for a better tomorrow. Without holding himself back, he worked for the benefit of all of us. We will greatly miss his knowledge, experience and calmness in making difficult decisions.

Everyone who knew him and who worked with him received the news of his sudden death with grief in their hearts. This is the only battle he failed to win. We will remember him and he will live forever in our hearts.

It's up to us to pick up where Vladimir has tragically left off.

Dear Vlado, May God bless you.

From the Serbian Haemophilia Society

The EHC expresses its most sincere condolences to the family, friends and community members of Vladimir.

Feature Articles

Researcher spotlight: Dr Mehdi Osooli

Interview taken by Raia Mihaylova, EHC Communications Officer

In this column, we explore the work of a researcher involved in the area of haemophilia and other rare bleeding disorders. For our final issue of the year, we are excited to introduce Dr Mehdi Osooli - epidemiologist at the Center for Thrombosis and Haemostasis, Skåne University Hospital in Malmö, Sweden. He got involved in the area of haemophilia by chance and considers himself lucky to have done so. In this interview, he shares many interesting research findings he has worked on and explains his vision for even better collaboration in the field of data collection.



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

I am an epidemiologist working now for seven years at the Malmö haemophilia treatment centre. I mostly look at long-term outcomes amongst people with haemophilia; I do have some other lines of research that I continue doing from my previous collaborations and work, but it is not relevant to bleeding disorders. So, as I said, my main research area is long-term outcomes among people with bleeding disorders, more specifically those with haemophilia – factor VIII and IX deficiency. We look at their joint outcomes, health-related quality of life and other determinants of how their treatment affects their health in the long run. We use registries – the Swedish registry – as well as international ones to look at these outcomes.

How did you become involved in this field?

In 2011, I was looking for opportunities to do my PhD at Lund University. I found a PhD position at the haemophilia centre, which works closely with the University, and I applied for it. I was not selected the first time around but I was persistent – I contacted the department again, asking if I could get involved in the research – luckily, they accepted. At that time, they were planning a large international registry project and they needed someone to take care of it, to establish it and to work as a project manager for that research. I took on that responsibility for my PhD work and I felt very lucky. Later on, I understood how significant this treatment centre is in the world, with years of history, initiating prophylaxis and so on. But back then, I didn't know that much, I was just quite excited to get a PhD. This is an honest answer. ☺

What does your average day look like?

My typical day is working with data obtained from our centre and our collaborating centres. I spend a lot of time reading recently published papers and my textbooks so as to improve my epidemiology knowledge and skills to do further research. I also use my time to interact with other researchers, many times contacting them to ask if they are interested in a collaboration.

What do you hope the impact of your work will be? What do you see as the end result/conclusion you would like to reach?

Ideally, we would like the results of our research to be used to inform policy-makers/payers on the optimal treatment for people with haemophilia. We have a great opportunity, using this long-term registry, because we are able to look into all the effects of treatment and follow up with patients. That is not easy and we want to use this opportunity to inform and to change practice where necessary.

What keeps you awake at night? (Any challenges from the day, a case that is not resolved...)

Apart from my daughters, who are both under the age of four, I often get really excited about a new research hypothesis that I have and I cannot stop thinking about it. I start searching for published papers on the topic, writing emails to people – asking what they think and if they are interested in further investigating. I am in touch with several well-known researchers and we will hopefully form research on certain populations that have not been well-investigated, perhaps those with mild haemophilia or carriers of haemophilia.

You have been involved in various research projects. Can you name one thing that really surprised you as a finding, or a situation where you set out and expected certain results but it turned out completely differently?

Yes, I was quite surprised in the case of researching joint outcomes among carriers of haemophilia, on which I have been recently working. General opinion and logic say that we do not expect frequent joint issues among haemophilia carriers, as most of them usually have a normal range of coagulation factor. However, in our and a few other studies, we discovered that haemophilia carriers actually are more likely to experience joint issues than the general population and are more hospitalised for joint problems. This is one finding that was pretty surprising and we have to further investigate that.

As you mentioned, during your PhD, you were actively involved in developing an international register to monitor treatment outcomes among people with haemophilia. Especially now, with so many novel therapies coming out, why is such a registry so important? What are some challenges?

The study that we started back in 2012 was an international registry focusing on people with moderate to severe haemophilia A. With that research, we aimed to investigate the outcomes of different treatment practices from various places, including treatment centres in Africa, Turkey, Scandinavia and other countries. I was involved in that research from the very beginning and it was really challenging. With the numbers of people at each treatment centre being nationally small, you really need to establish an international project that covers many treatment centres. This also helps in gaining a wide range of information about people with haemophilia, in terms of treatment background and health outcomes, which helps to generalise your findings for the larger overall population of people with haemophilia. This type of research is also important because it helps to design better treatment practices. The clinical trials that are usually used for defining or deciding which treatment is superior to another do not really take into consideration real-world practice – what happens in treatment centres later on when the doctor and the person with haemophilia who is receiving the treatment is not within the tight, controlled setting of the trial?

One of the challenges with including various settings and locations is that everywhere they have different routines and ways for following up with patients, different motivations for collecting data, and the quality of data varies greatly between centres. We also have challenges when it comes to data protection rules between countries and even between centres within a country.

How and where do you even begin with such a project?

Usually, researchers and clinicians are closely connected. One way to start out is by trying to find who else is investigating these outcomes and approaching them, describing the study and asking if they are

interested in participating. Mostly, the response is positive and being able to collaborate makes the process easier.

What is one thing about haemophilia that surprised you?

There are a lot of things that surprised me! Haemophilia is one of the most expensive health conditions in the world to treat. Luckily, treatment works very well, but of course, at a very high cost. The surprising point for me is that after a few years of adapting to treatment, there will be some people with haemophilia that stop following their treatment. For example, this may happen during teenage years, or some other transitions in life. This is a bit unexpected with such expensive and effective treatment, and we don't want to lose the opportunity to protect peoples' health and to miss good treatment practices.

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

In the near future, but this is something that has actually already started, we will use big data for people with haemophilia as well, just like for other disorders. I very much look forward to seeing how we can use big data for this population. Big data means using multiple and huge sources of information to look into a specific question. Combining multiple databases, patient databases, and online databases leads to the questioning of how all these different sources can be combined in the near future to gather better research outcomes.

If you had not been working in the field of epidemiology, you would have been working on...?

Perhaps it is not too late, one can always switch if one finds something one is more passionate about. If I weren't an epidemiologist, I would perhaps be an entrepreneur working on everyday challenges in society to find innovative solutions. This is something I tend to do today but I would be more than happy to extend this beyond research.

So you are about finding solutions overall?

Yes! It is something I am very passionate about.

Is there anything you would like to add?

I have decided to set up several important research projects in the coming years to be conducted across treatment centres on a global level. I have started contacting people doing similar research – collaboration is the most important thing in research, from which we can all benefit and I push for that. Together we can find better solutions and to assure that people with haemophilia and other bleeding disorders have higher health-related quality of life. That is why I push for more collaboration in research.

The European Pillar of Social Rights: what you need to know

By Yordan Aleksandrov, Consultant, Rohde Public Policy

Background

The vision for the European Pillar of Social Rights was first introduced in Jean-Claude Juncker's State of the Union speech in 2015. A year later, the European Commission presented a preliminary outline of what should become the European Pillar of Social Rights. Following a public consultation, the Commission delivered a [final version](#) of the text in April 2017. Later in November, the text was [jointly endorsed](#) by the European Parliament, the Council of the European Union and the Commission at the Social Summit for fair jobs and growth in Gothenburg.

Objective

The Social Pillar is intended to drive forward a social Europe for all European citizens. It aims to strengthen the social acquis¹ and deliver more effective rights to citizens. The overall objective of the Pillar is to contribute to social progress by supporting fair and well-functioning labour markets and welfare systems.²

Composition

The European Pillar of Social Rights is about delivering new and more effective rights for citizens. The text is divided into three chapters: (I) Equal opportunities and access to the labour market; (II) Fair working conditions; and (III) Social protection and inclusion. These chapters build 20 key principles, two of them are in relation to health:

- *Healthy, safe and well-adapted work environment and data protection* (principle 10, Chapter II)
- *Everyone has the right to timely access to affordable, preventive and curative health care of good quality* (principle 16, Chapter III)

Implementation

Implementing the ideas of the European Pillar of Social Rights is a joint responsibility at the hands of Member States, social partners and civil society, and the European institutions. To this end, the European Commission has a crucial role in providing the relevant framework and guidance. The implementation of the Pillar will also have an [online social scoreboard](#) to monitor trends and performances across EU countries.

The Commission has already provided directions to legislative and non-legislative initiatives related to work-life balance, the information of workers, and access to social protection and working time. In April 2017, the Commission put forward a **proposal for a Directive on Work-Life Balance for Parents**

¹ The social acquis is the part of the *acquis communautaire* that includes the body of EU laws, principles, policy objectives, declarations, resolutions and international agreements defining the social policy of the EU.

² *European Pillar of Social Rights: Proclamation and Signing*, available at: <http://www.consilium.europa.eu/en/press/press-releases/2017/11/17/european-pillar-of-social-rights-proclamation-and-signing/>

and Carers. Next to formally recognising carers, the proposal includes a minimum five-day paid leave per year. Rapporteur in the European Parliament for the dossier is David Casa (EPP, Malta). In December 2017, the Council of the European Union will discuss the proposal, while an official hearing in the European Parliament will be organised in quarter 1 of 2018. It is also envisaged that the text is finalised and adopted by mid-2019.

Are voluntary frameworks a realistic solution to increasing access for haemophilia patients?

By Yordan Aleksandrov, Consults, Rohde Public Policy

Since its founding tenets were established after the Second World War, the European Union (EU) has walked a delicate balance between the integration of competences and the cooperation amongst Member States, guided by the principle of subsidiarity³. While this complex, but prosperous, equilibrium has been studied and debated for more than 50 years now, another area of collaboration in a rather sensitive field is emerging. With novel health technologies steadily approaching on the horizon, joint negotiations of medicines purchasing and voluntary collaborations in this field are fighting their way into a new style of Member-State-driven partnerships. This new version of cooperation illustrates a pragmatic use of another European principle: the principle of solidarity. However, the main question remains: are these agreements effective? Could they be extended? What are their consequences?

What are Joint Procurement Agreements (JPA)?

In EU jargon, Joint Procurement means combined purchasing of goods by two or more contracting Member States, so that only one tender is published on behalf of them all.⁴ Following the H1N1 pandemic, the European Commission approved in 2014 a JPA, which enables EU countries to procure pandemic vaccines and other medical countermeasures as a group.⁵ As of October 2017, 24 EU countries have signed the agreement. Since then, JPAs in the field of medicines are provoking huge political interest.

Are JPAs effective?

Generally speaking, yes, there are indications that JPAs are effective. This can be seen, for example, through the one-year assessment of joint procurement of pharmaceuticals in the public health sector in Jordan, which has indicated in 2009 that such an agreement has the potential to reduce expenditures for the purchase of medicines and provide treatment continuously throughout the year.⁶

What is voluntary cooperation?

The BeNeLuxA collaboration is a prime example of voluntary cooperation in the field of medicines. Launched in 2015 by Belgium and the Netherlands and later joined by Luxembourg (2015) and Austria (2016), the initiative aims at state collaboration in a range of areas such as horizon scanning, information sharing, Health Technology Assessment (HTA), and pricing and reimbursement in the field of rare diseases. The ultimate goal would be to ensure timely access and affordability for patients. Since then, these types of initiatives have been growing in numbers in Europe.

Is the BeNeLuxA initiative effective?

In the EU, and within the framework of the BeNeLuxA agreement, the countries have so far jointly conducted negotiations with the US-based pharmaceutical firm Vertex regarding the reimbursement of Orkambi, but these talks failed. Orkambi is indicated for the treatment of a specific form of cystic fibrosis, a rare inherited disease that has severe effects on the lungs and the digestive system (it is also

³ The principle of subsidiarity is defined by Article 5 of the Treaty of the European Union. It states that “*in areas which do not fall within its exclusive competence, the Union shall act only if and in so far as the objectives of the proposed action cannot be sufficiently achieved by the Member States.*”

⁴ <http://www.pharmexec.com/joint-procurement-moves-eu-agenda>

⁵ http://europa.eu/rapid/press-release_IP-14-418_en.htm

⁶ <https://www.ncbi.nlm.nih.gov/pubmed/19695398>

known as “CF”). As the BeNeLuxA cooperation is still in progress, this development shows that for the time being, all sides are empty-handed, as Belgian and Dutch CF patients will not obtain the best possible care, while Vertex is denied access to the market.⁷

The political reactions

Given the potential of such agreements in enhancing the leveraging position of Member States during price negotiations of medicinal products, the JPAs and voluntary cooperation initiatives have strong political support.

With innovative medicines emerging, the European Parliament and EU Member States are expressing concerns about the sustainability of health systems and often underline the competence of local governments in health care system organisation. Because innovative medicines often come at a price, it should be noted that the political support behind these initiatives is, for the moment, still hugely concentrated on prices rather than on patient access. However, this “price above all” approach seems to be slowly losing its grip, as Members of the European Parliament have expressed concerns that making the pharmaceutical industry ‘the villain’ is not productive for patient access. Price is only one of many factors playing into the access to medicines debate. In fact, several reports from the European Commission have indicated that price reduction techniques are unlikely to increase access, particularly in less-developed markets in Eastern Europe.⁸ To this end, this debate needs to encompass all angles. It is in this context that the European Haemophilia Consortium (EHC) are increasing their efforts in addressing the problem without omitting all of its crucial aspects.

Consequences for people with haemophilia

For people with haemophilia, this trend is positive, as currently several new-generation treatments are being developed (e.g. gene therapies). Joint collaborations could potentially accelerate access to these innovative therapies if a framework can be found that puts access first and companies are aligned on the benefits of that framework. Common cooperation in the areas of HTA for example and exchange of information could be favourable for greater quantitative purchase of FVIII and IX concentrates where inevitably, with new innovative options on the market, their prices are likely to fall. This is something, which indeed, is also at the heart of the recently launched EHC PARTNERS* programme in the European Parliament, which aims at:

1. Improving access to treatment for people with haemophilia in countries below the minimum level of treatment as recommended by the Council of Europe, and improving standards of care for people with haemophilia within these countries;
2. Involving clinicians and patient representatives in the procurement process, and making procurement a national process, to seek to improve health care systems and ensure their sustainability.

* Procurement of Affordable Replacement Therapies - Network of European Relevant Stakeholders

⁷ Beneluxa cooperation for medicines – Belgium and the Netherlands Jointly Decide not to Reimburse Cystic Fibrosis Medicine of Vertex, available at: <https://www.vbb.com/insights/corporate-commercial/corporate-commercial/beneluxa-cooperation-for-medicines-belgium-and-the-netherlands-jointly-decide-not-to-reimburse-cystic-fibrosis-medicine-of-vertex>

⁸ European Commission, Study on enhanced cross-country coordination in the area of pharmaceutical product pricing, 2015, available at:

https://ec.europa.eu/health/sites/health/files/systems_performance_assessment/docs/pharmaproductpricing_frep_en.pdf

JPAs and voluntary cooperation so far

- Voluntary cooperation between 24 EU governments on the Joint Procurement of pandemic vaccines and medical countermeasures.
- An EU-supported project called “Healthy Ageing – Public Procurement of Innovations (HAPPI)” was set up in 2012 to enable public health purchasers to collaborate on detecting and purchasing innovative and sustainable solutions to facilitate healthy ageing.
- The European Commission has been asked to investigate the possibility of organising Joint Procurement for hepatitis C treatments.
- In June 2016, representatives of Bulgaria, Croatia, Estonia, Hungary, Latvia, Romania, Serbia, Slovakia, Slovenia and the former Yugoslav Republic of Macedonia signed a declaration that they would collaborate on issues affecting access to medicines.
- In April 2015 Belgium and the Netherlands, later joined by Luxembourg, signed a memorandum of understanding to exchange information on and, in selected cases, jointly negotiate prices for medicines for rare diseases, particularly orphan drugs.
- In June 2015, Nordic pharmaceutical forum was set up with the intention of achieving collaboration between purchasers of medicines in Denmark, Iceland, Norway and Sweden, sharing experiences and jointly tackling common challenges around new and expensive medicines, horizon scanning and supply chain safety.
- In October 2016, Romania and Bulgaria signed a joint procurement agreement to negotiate medicines pricing.
- In 2017, the Valetta declaration was signed. Six southern Member States signed a common declaration, aiming to enhance their cooperation and jointly negotiate with the pharmaceutical industry on drug pricing issues.⁹ It is likely that the collaboration will also expand to HTA, information sharing and horizon scanning, similar to the BeNeLuxA initiative.
- On 26th October representatives of Poland, Croatia, Slovakia, Slovenia and Lithuania met in Vilnius to discuss preparations for common negotiations with pharmaceutical manufacturers to jointly purchase innovative expensive medicines.

⁹ Malta, Cyprus, Greece, Italy, Spain and Portugal

Currently around two thirds of EU Member States have signed cooperation agreements. It seems that at this stage, the agreements do not produce substantial concrete results, with a few exceptions.

Issues related with voluntary cooperation

Indeed, in Europe it is clear that the idea and the willingness of joining forces exist. However, it seems that today, initiatives around medicines are approached more in a 'competitive' light - i.e. **which country would sign them first** - rather than in the light of addressing urgent need and correct implementation. This is confirmed by the scarcity of any substantial information in the public domain on this topic. One particular example always comes to mind: Bulgaria and Romania have signed a memorandum of understanding in October 2016. Still, this agreement has yet to be ratified by both of the countries' Parliaments.

As is the case with the European integration process, the *implementation* of provisions is the key to success. It is clear that if properly implemented, JPAs and voluntary negotiations in the field of medicinal products could improve health care sustainability and improve patient access to treatment without infringing on the principle of subsidiarity. However, there is still a clear need for coordinated support in the field. In this sense, the EU could play an important role in politically bridging the gap between Member States with the end goal of improving access to lifesaving therapies. This presents an opportunity for the EU to build a closer relationship with its citizens.

Today, the EU is at a crossroads. The Union could be more than a market, but it needs to deliver to citizens in terms of concrete advantages to them for being part of the "club." Although health care is a delicate field, with Member States fighting to preserve their sovereignty in this area, the JPAs are encouraging moves towards voluntary cooperation, which could be concrete tools to address patients' needs across Europe.

Mindfulness

Written by Lisa Miller, member of the UK Haemophilia Society, EHC UK NMO

Be kind to yourself. That's one of the biggest – if not the biggest – things I learned from a recent mindfulness course I attended at the hospital that cares for my son.

What is mindfulness? Essentially, it is to be aware of the present moment without judging or ignoring what is happening around us, but also letting go of the pressures that this might bring. It is not getting caught up in the moment but instead standing back, recognising and observing our feelings, not feeling guilty or hard on ourselves about how we might feel but more importantly, learning the skills necessary in order not to be affected by their negative thoughts. Not an easy task, but one that felt like it would be hugely valuable in helping me cope as best I could with difficult situations when caring for my son – or in fact, in any part of my life.

So, this definitely sounded like the course for me! Let me first explain why, with a little bit of background about my son and his haemophilia.

My 12-year-old son has moderate haemophilia A with inhibitors. The inhibitor was like a second diagnosis to us and in many ways, we were a lot less prepared for it than for the haemophilia diagnosis itself when he was five days old. I always knew I was a carrier and understood the genetics behind it. Isaac was first diagnosed as a mild haemophiliac with factor VIII levels around nine. The first 11 years were scattered with the uncertainties and unpredictability of a haemophilic child living with treatment only on demand. But apart from worrying about accidents, it actually really wasn't that big a deal. Treatment is so great these days and we had a wonderful team at the hospital. We fell into a way of dealing with having to be flexible at times, changing plans sometimes around a bleed, missing a little school, hospital stays – but it really wasn't that bad. Treatment was working well and we knew where we stood with the diagnosis.

When Isaac's levels were re-tested at 11 years, he was re-diagnosed as a moderate haemophiliac with factor VIII levels closer to 3-4. With 49 doses of FVIII under his belt and about to head off to secondary school, it was decided that he should move to prophylaxis. He learned to self-infuse over the summer holidays and we had around three months of him treating himself with no bleeds. Great!

Then life changed for us all. When we couldn't stop a wobbly tooth from bleeding for over a week, his regular blood tests were brought forward and we found out he had no FVIII in him at all. He had developed an inhibitor. I would be lying if I didn't say life suddenly got a lot more difficult, worrisome and, to be honest, frustrating. Frustrating because the treatment wasn't working at first and then, frustrating because we had to inject every day of the week and as part of that, frustrating because the process of the injections didn't always go well.



Lisa (left) with her family and other participants at the EHC 2017 Inhibitor Summit

We have had excellent support from our specialist nurse, consultant, physio, dentist and psychologist but I was really finding it difficult to find my way through those mornings where Isaac just didn't want to do his injection. The worry of Isaac not having his factor and therefore, having a bleed, being late for school, missing school altogether, me being late for work, the disruption to my 10-year-old daughter's morning routine, the tension it would cause my husband and me and just the downright frustration of trying to understand "why won't you just do the injection today!?" You might know that feeling!

And of course, he never knew why. He is only 12 years old and one minute he was self-treating twice a week and avoiding bleeds and then suddenly, developed the inhibitor to the very treatment he was injecting himself with to keep himself safe. It's a lot for an adult to understand but for a child? We were asking a lot from him and yet, we had no choice and we needed him to cooperate. Otherwise, things were never going to have a chance of improving. However, us losing our cool on difficult mornings was the last thing we needed – and it never helps anyway!

With home life being disrupted on a fairly regular basis, his psychologist suggested I attend a "Nurturing Parents" course that she was running at the hospital – a course on mindfulness specifically designed for the well-being of parents who are caring for children living with conditions that affect their physical and mental health.

I had read a bit about mindfulness in the past, practiced a little meditation and also, attended a couple of one-off seminars about the benefits of mindfulness but this felt like the right time to learn how it could help me manage my way through the difficult days in a less stressful way.

Over the course of eight weeks, a small group of us would come together each week to learn, share and practice. And the aim of the course? Using mindfulness techniques to help identify signs of stress and anxiety early on so that they can be addressed quickly and before they take control over our emotions.

I learned a lot from this course and just wanted to share a few little insights that I had or skills that I learned that I feel help me (sometimes!) on the difficult days. I am not an expert. I don't come with evidence to show how this works. I can't say it will work for everyone but, I can say what worked for me and hope that something you read might help you too.

One of the first steps in being more mindful is recognising what causes our stress and how we manage it. We talked about the concept of "flipping our lids" and how we become disconnected from what is happening around us when this happens. Dan Siegel on YouTube is the man to explain this using his hand model of the brain. It is a helpful visual to think about how we can sometimes become disconnected from our emotions and react without thinking clearly. By trying to be kind to ourselves in these moments, we have a better chance of not becoming overwhelmed by the emotions – of not flipping our lid.

The very essence of mindfulness is not to put pressure on ourselves but to be kind to ourselves and others. So, no judgements were made by how little time we might have found to practice that week or, how we might still have gotten frustrated with a situation with our children since we last met. Allowing us to accept that sometimes this happens but also encouraging us not to criticise ourselves and not feel guilty about it was a great relief. I am sure we all do the above when we reflect back on a situation we perhaps didn't handle as well as we would have liked.

One quick tactic we were taught to keep the stress under control when in a difficult situation is the “three step breathing process,” which you can do with eyes open or closed and should take just a few moments:

1. Stop whatever you are doing. Maybe change how you are sitting or standing. Become aware of how you are feeling – no pressure to change this, just observe your thoughts and feelings.
2. Focus your attention just on your breathing – no need to change your breathing, just try to think about your breath.
3. Now focus on your whole body – again, no need to change anything, just think about how your body feels.

This has been enormously helpful for me. I try not to become stressed when the injection isn’t going as well as it does most days. I try to sit and breathe... think about how I am feeling... how Isaac might be feeling... what is happening at that moment in time... how I can help move the situation along – rather than just shouting about being late for school and how important the injection is!! It never helps. He knows all that anyway and we just all end up feeling bad when that happens.

Mindfulness is not only about finding time to “practice.” It’s also about just being more “mindful” of the everyday things we are doing. Paying close attention to what we are doing at that moment in time is a great way to slow down, focus the mind a little, stop worrying about all that is ahead... whether it be eating an apple, washing up, taking a shower, driving the car, brushing your teeth, listening to your child read and so on – just focus on the present moment. This too is useful at those times when things are not going the way you want them to.

Mindfulness can also encompass how we communicate with our children and others around us. Children feel enormously empowered when they know they are being listened to. Staying calm at difficult times, looking at our children, really listening – properly listening without interjecting, without trying to fix things, without second guessing what they are trying to say, just listening to every single word and hearing what they are really saying. This can be really beneficial in better understanding what our children need from us and how we might be able to better meet those needs. It’s surprising what we can miss when we are actually thinking about something else when listening!

Having said it’s not just about finding the time to practice, this does, of course, play a part in mindfulness and so, we were encouraged to try to find time each week for a little mindfulness practice. We can do this in different ways – mindful breathing, mindful movement, mindful sitting. For me, it doesn’t really matter which you choose, it is just about giving yourself permission to take a few moments out of life and be in the present moment.

I set out to learn skills that would help me emotionally when sitting with my son, early in the morning, trying to encourage him to do his injection or, let me do his injection. I have learned how important it is to notice how I feel and behave on the difficult days and use some mindfulness techniques to help me stay in the moment without becoming too stressed or emotional. I now know that I need to be kinder to myself in those moments. Take a pause. Step back. Breathe. Observe and then, try to work with my son to achieve our daily injection regime.

As I said before, I am no expert in this, but having learned a little about mindfulness, I can really see its uses for us parents (and others) trying the best we can to find our way through the challenges life brings us when caring for our boys. Be kind to yourself and acknowledge what a great job you do every day. Learn from the difficult days but by being gentle with yourself – observe what the struggle was and think about “how I can improve that the next time without passing judgement”.

News from our corporate partners

The EHC would like to acknowledge and thank the 2017 corporate partners of the EHC Round Table of Stakeholders programme, which promotes dialogue and exchange between all stakeholders. In this spirit, we are pleased to share their news with readers in this section.

Platinum partners:

Novo Nordisk

Pfizer

Roche

Shire

Sobi

Gold partners:

Bayer

CSL Behring

Silver partners:

Alnylam

Biotest



together we can change haemophilia

"We must help one another,
and accept that it is ok
to ask for help too"

Sine Lyons
Mother of Carl, who has haemophilia A
Denmark

Changing Haemophilia® was established to help people living with haemophilia to live a life with as few limitations as possible.

We work closely together with haemophilia organisations, as well as patient organisations like the European Haemophilia Consortium (EHC) to support, advocate for, and create better access to diagnosis and multidisciplinary care.

Together, we aim to change perceptions and behaviours by raising awareness, and supporting projects so that people with haemophilia can experience the freedom to live life to the fullest.

Learn more at novonordisk.com/changinghaemophilia

Improving joint health and portability for haemophilia patients

To help people with haemophilia live life with as few limitations as possible, our latest campaigns focus on the importance of exercise for joint health and mobility, and enjoying life without feeling limited by the need to keep treatment under refrigeration.

The videos are part of our portability campaign Little Epic Adventures, which aims to encourage people with haemophilia to enjoy their lives, and embark on new adventures, without feeling constrained by factor refrigeration.

The animations demonstrate the benefit of frequent exercise. Featuring members of the haemophilia community, each person explains how their preferred form of exercise helped increase their mobility, reduce their bleeds and improve their joint health.

Follow Novo Nordisk to explore our latest videos:

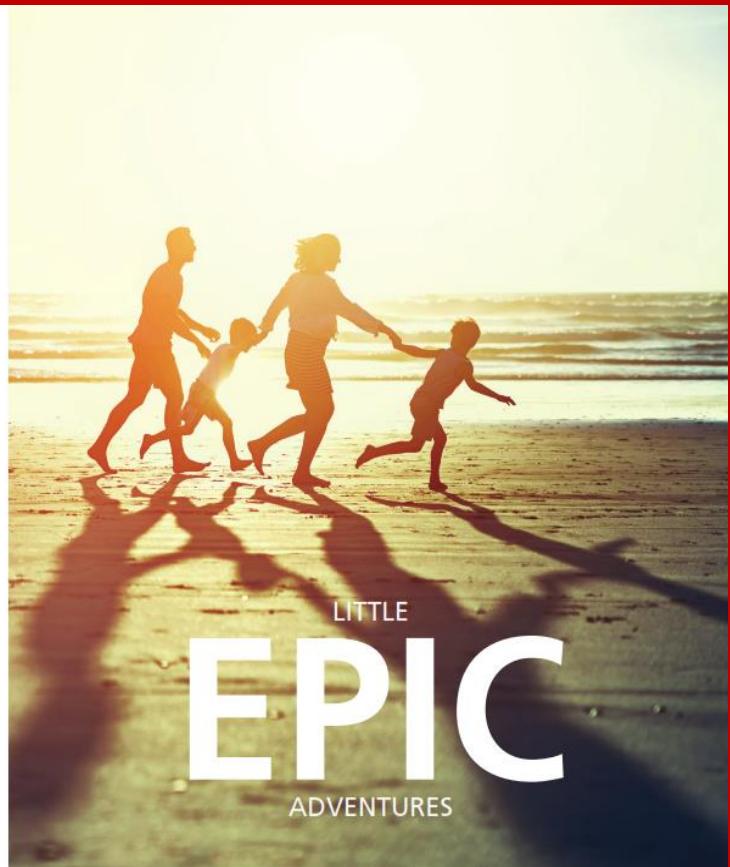
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Or watch the videos here under Changing Haemophilia®
<http://video.novonordisk.com>

JECOREI LYONS
Jecorei lives in the USA
and has Haemophilia A



Changing Haemophilia® is a trademark owned by Novo Nordisk Health Care AG and the Apis bull logo is a registered trademark of Novo Nordisk A/S.

HQMMA/CH/1217/0198. Date of preparation: November 2017



Partnering to Change the World for People with Haemophilia

EHC Newsletter 2017

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

Haemoassist® 2 Patient App*

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

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

Miles for Haemophilia: Your Personal Best**

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

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

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

Physiotherapy School for Haemophilia

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

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

Partnering to Change the World for People with Haemophilia

Pfizer Ultrasound Programme

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

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

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

EHC Website and Learning Portal

Part of the EHC's work is to educate their member organisations through close collaboration to ensure a strong collective voice for people with rare bleeding disorders.⁴ Pfizer Haemophilia understands the importance of disseminating this information, and therefore supported the recent update and revision of the [EHC Website](#) in 2017 to enhance this valuable resource. Pfizer Haemophilia provided further commitment to patient advocacy groups by providing an educational grant for the development of the EHC Learning Portal. The [EHC Learning Portal](#) is an online tool, designed to support the dissemination of education around principles of health technology assessment and health economics. The tool has been designed as an interactive resource to allow for self-directed learning at a pace that suits the user.

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

WFH Twinning Programme

Pfizer Haemophilia is proud to be the exclusive sponsor of the [WFH Twinning Programme](#), encouraging exchange of experience, skills and resources related to haemophilia treatment between countries. In 2017, Pfizer Haemophilia celebrated the 16-year anniversary of the programme's launch, during which time 212 partnerships across 111 countries have been established.⁵

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

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

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

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

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

These programmes are initiated and funded by Pfizer



We are Roche

The Roche group, including F. Hoffmann La Roche Ltd, Genentech and Chugai pharmaceuticals, has been committed to improving lives since the company was founded in 1896 in Basel, Switzerland. Today, Roche creates innovative medicines and diagnostic tests that help millions of patients globally for conditions of significant unmet need. Our combined strengths of pharmaceuticals and diagnostics under one roof have made Roche the leader in personalised healthcare – a strategy that aims to fit the right treatment to each patient in the best way possible.

Roche is active in over 100 countries and in 2016 employed more than 94,000 people worldwide. The company is strongly committed to patient focused scientific innovation and in 2016, invested CHF 9.9 billion in Research & Development.

With more than 140 medicines on the market and twenty-nine medicines included in the World Health Organization Model Lists of Essential Medicines, we are proud to be serving patients around the world. Our medicines are indicated for a broad range of disease areas including: Oncology, Haematology, Haemophilia, Neuroscience, Infectious diseases, Immunology, Cardiovascular and metabolic disorders, Ophthalmology.

Roche's commitment to haemophilia

The Roche Group's involvement in haemophilia-related research stretches back several decades. In 1984 research scientists who were members of the Department of Molecular Biology and the Protein Biochemistry unit at Genentech (a member of the Roche Group) published groundbreaking research related to the structure and genetics of human Factor VIII as well as the expression of Factor VIII from recombinant DNA in the scientific journal *Nature*¹. Genentech became the first company to produce recombinant Factor VIII².

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

Our commitment to patients is demonstrated in many ways throughout the company and is fundamental to what we believe to be Roche's most significant contribution to society: to create, produce and deliver innovative solutions of high quality for unmet medical needs. Our shared commitment to patients makes us eager to listen, understand and collaborate with the haemophilia community to improve patients' access to innovative treatments and services. At Roche we are convinced that an open dialogue and transparent exchange of information among partners in the healthcare community is vital to advancing access and healthcare delivery to patients. Building and sustaining relationships with patient groups is an important way for us to emphasize our commitment to patients and to society as a whole. For more information on Roche in haemophilia, please visit www.roche.com/haemophilia

Roche continues to Reimagine Haemophilia A during the 2017 EHC Consortium

We had the privilege to participate in the EHC Annual Conference in Vilnius, Lithuania. It was a unique experience and an opportunity for our team to meet and engage with members of the EHC community in interesting and insightful conversations, listen and share a common vision for the future of haemophilia. Through an interactive corporate booth, we were able to meet and engage with participants of the conference in many ways.



Firstly, as recent years have been marked by the advancement of science and the opportunity of a new future for the haemophilia community, we wanted to better understand what their aspirations were around a life without bleeds by asking them: **"What could a life without bleeds mean to you, your family and the community?"** We gathered quotes that inspire us to continue supporting research and to develop meaningful partnerships that help the community reach their goals. Those comments were written on "postcards" that were stuck on the booth wall.

"Life without bleeds would be life with more activities, trips, experiences life with less pain. Less time thinking and being aware to your body as pain centered and change conception about body and myself"

"More smile, more calmness, more opportunities."

"Freedom to plan, reassurance for the future, better freedom, security"

Adding an extra dimension to their insights and by way of visually representing participants' thoughts, we used a "word map" that captured their "One Word" about a life without bleeds. This visual representation evolved through the conference, revealing that **"Freedom"**, was the emerging thought and that represented aspirations of the community.

possibilities amazing opportunity
magic control reborn potential
freedom health privileged security lucky easier
protection reassurance



We also proposed an educational component, which enabled participants to increase their knowledge of the FVIII mechanism of action within the coagulation cascade. This educational activity was delivered through a "Virtual Reality" experience where participants saw the mechanism of action through 3-D glasses. Participants' feedback was very positive as it brought this complex mechanism of action to life.

Lastly, Roche held an educational symposium focussing on sharing the recent advances in Haemophilia A management and potential novel treatments. It included polling questions with the audience on how these novel agents would address unmet needs. The symposium was chaired by Brian O'Mahony, President of EHC, who was joined on stage by two esteemed faculty members, Dr. Kate Khair from Great Ormond Street Hospital (London, UK) and Professor Cédric Hermans from Saint-Luc University Hospital (Brussels, Belgium). The presentations were followed by a panel discussion and incorporated questions from the audience.

EHC[®]

The Future of Haemophilia A Management
Can New Therapeutic Approaches Achieve Community Aspirations?

Alfa Room, The Radisson Blu Hotel Lietuva
Vilnius, Lithuania

Brian O'Mahony (Chair)
Kate Khair
Cédric Hermans

This presentation contains information on investigational products that are not yet approved or licensed for the treatment of patients with haemophilia A or any other medical condition.
This symposium is not intended for physicians practicing in the USA.

NPIACE/1709/0020e

Roche is proud to support and work in partnership with EHC and the haemophilia community in order to address the existing challenges. Key principles of our work with patient organisations are integrity, transparency, trust and mutual respect.

Let's continue to Reimagine Haemophilia A Together



Unmet needs require unmatched commitment.

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

For more information, please visit shire.com



Young stars take on experts at EHC Youth Debate

Youthful enthusiasm and emotion took on experience and expertise in the Youth Debate at the European Haemophilia Consortium's 2017 annual conference in Vilnius in October.

Sobi again donated back its EHC symposium slot for the Youth Debate, bringing young patient advocates head to head with leading experts who are required to take a hard opposing line, in a session which has become a highlight of the annual EHC conference.

Amanda Bok, CEO of the EHC, says the debate is part of the broader EHC Youth Leadership programme.

"It's a good format because it's compelling, it's high energy, it's fun and it's empowering for the people who will be our future leaders," she says.

Phil Wood, Head of Haemophilia, Commercial, at Sobi says the Youth Debate allows young leaders to practise discussing topics that are highly relevant for the haemophilia community, improving their argumentation skills against tough opponents.

This is the fourth year that Sobi has donated back its EHC symposium slot for the Youth Debate.

DEBATE 1

Patients should have the final say in product selection



ROBBERT-JAN 'T HOEN,
youth debater, The Netherlands

Robbert-Jan 't Hoen argued for the affirmative. He stated that the quality of life of people with haemophilia should be given more weight in medication decisions, and that better outcomes should be prioritised rather than simply cost.



DR PAUL GIANGRANDE,
chair of the EHC Medical Advisory Group, UK

Dr Paul Giangrande, for the negative, countered that doctors were better informed and better placed to take objective decisions about medication. Doctors also shared responsibility for ensuring equitable treatment for all patients, and bore the ultimate medico-legal responsibility.

The audience
voted with the
negative

DEBATE 2

Payers should consider haemophilia as a special case



VALENTIN BRABETE,
youth debater, Romania

Valentin Brabete from Romania, for the affirmative, said that people with haemophilia who receive appropriate treatment could live close to normal lives, contributing to society and achieving their full potential. Failure to treat properly leads to higher direct and indirect medical costs, as well as greater dependence on welfare and social assistance. A timely decision by the payer avoids higher costs later.



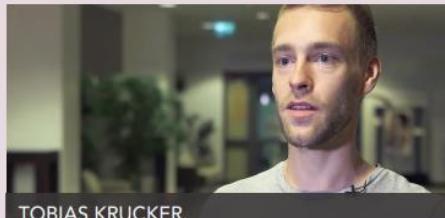
MARK SKINNER, member of Medical and
Scientific Advisory Council of the US
National Hemophilia Foundation

Mark Skinner, for the negative, countered that payers must balance demands on limited budgets, with expert committees using objective information to make well-founded decisions to achieve the greatest good.

The audience
voted for the
affirmative

DEBATE 3

Pre-natal selection should be available to all in the bleeding disorders community



TOBIAS KRUCKER,
youth debater, Switzerland

In the final debate, Tobias Krucker from Switzerland argued for the affirmative that as a 25-year-old man with haemophilia A, he is reaching the age where he is thinking about having children, but his diagnosis creates a dilemma. If he were to decide only to have sons, his condition would not be passed on, creating a future without haemophilia for his children and grandchildren. With the high costs of treatment, such a decision would mean major financial savings for society and remove the risk of future cutbacks resulting in a poorer outcome for his descendants.

Emotionally, he told how his grandmother once spoke of her guilt at passing on the condition to Tobias' mother and uncles, and how he would feel similarly guilty about his children or grandchildren if they also had haemophilia.

"This is a controversial, personal decision – nobody should be forced to carry out pre-natal selection, it should be up to me and my partner – but it should be an option for future parents."



DR DAN HART, EHC Medical and Scientific
Advisory Group, UK

Dan Hart acknowledged that Tobias made a powerful and emotional case, but stated that in the UK, there were very few cases of prospective parents requesting screening for haemophilia compared with other genetic diseases.

He also referred to elite cyclist Alex Dowsett, and a patient group in Bangladesh, showing what people with haemophilia could achieve. One of his final points addressed where widespread genetic screening could potentially lead, and the possible repercussions for those who did not screen in such a future.

In the closest
result of the night,
the negative won
by 48-52%

Hello Haemophilia a community for positive change

Hello Haemophilia, an online community officially launched at EHC 2017, is already bringing thousands of people together in a conversation about creating positive change in haemophilia.

Sponsored by Sobi, Hello Haemophilia is an online community for people living with haemophilia – patients, their families, caregivers, physicians, organisations.

Ana Palma, Community Engagement & Patient Access Lead, BeNeLuX at Sobi, says that by introducing unique perspectives into the community, Hello Haemophilia can help increase awareness and shape a better future for people with haemophilia.



"Hello Haemophilia is a digital space for sharing, listening, learning, collaborating, connecting, co-creating and so much more. No problem is too small to discuss on Hello Haemophilia – and no idea is too large.

Together, we can nurture these perspectives and co-create the

solutions needed to make a difference for our community."

Come say hello today, and join a community that's passionate about positive change.

hellohaemophilia.com
[facebook.com/
hellohaemophilia](https://facebook.com/hellohaemophilia)





Bayer's passion for patients

Committed to advancing the treatment of **hemophilia**

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



**Delivering on our
promises is what we do.**

**Unlocking the promise of
biotherapies is how we do it.**



*CSL Behring is proud to work together with
the European Haemophilia Consortium to help
ensure haemophilia patients' needs are met.*

Biotherapies for Life®

CSL Behring

Alnylam: Advancing RNA Interference (RNAi) Therapeutics

Who are we?

Alnylam was founded in 2002 to advance RNAi therapeutics as a potential new class of innovative medicines. Our pipeline has a strong focus on rare genetic medicines, with additional areas of investigation that include cardio-metabolic and hepatic infectious diseases.

Dedicated to developing new treatments for rare conditions

Alnylam is working to fulfill unmet clinical needs with a broad pipeline of investigational therapeutics based on our novel RNAi platform.

We are committed to serving patient communities through education and awareness, with a patient advocacy team dedicated to working with the bleeding disorders community.

Changing the thinking on haemophilia

At Alnylam, we are evaluating whether investigational RNAi therapeutics can help the body improve thrombin levels to accomplish the natural clotting process.

www.alnylam.com



Announcements

SAVE THE DATE!

EHC Leadership Conference 2018

7-10 June

Brussels, Belgium

The EHC is delighted to announce that the next EHC Leadership Conference will take place from **7-10 June 2018** in Brussels!

The purpose of the Leadership Conference is to bring together the full EHC community through **different key leaders and staff** from within your patients' communities to exchange and engage on shared issues and challenges. The objective of this conference is really to be as **interactive** and **engaging** as possible, and to provide you – our members – enough time to discuss the issues that matter to you and to benefit from each others' **strengths, experiences and best practices**.

A detailed programme and call for registrations will be sent out in the beginning of March 2018. For the moment – please save the date **7-10 June 2018**!



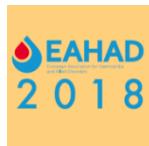


EHC 2018 events

- February 27** Round Table on Economics and Access, Health care Systems and Novel Therapies
Brussels, Belgium
Open to select participants
- April 6-8** Youth Leadership Workshop
Amsterdam, Netherlands
Open to NMOs only
- April 19** World Haemophilia Day event
Brussels, Belgium (TBC)
Open to all
- June 7-10** Leadership Conference
Brussels, Belgium
Open to NMOs only
- June 19** Round Table on Women and Bleeding Disorders
Brussels, Belgium
Open to select participants
- September 14-16** Tenders and Procurement Workshop
Location to be confirmed
Open to NMOs only
- October 5-7** 2018 EHC Conference
Brussels, Belgium
Open to all
- November 20** Round Table on Switching from Standard Therapies: Where do Novel Therapies Fit In
Brussels, Belgium
Open to select participants
- November 23-25** New Technologies in Haemophilia Workshop
Location to be confirmed
Open to NMOs only
- December 6-9** European Inhibitor Summit
Barretstown, Ireland
Open to NMOs only

Other events 2018

**February
7-9**



Annual Congress of the European Association of Haemophilia and Allied Disorders
Madrid, Spain
More information at <http://eahadcongress.com/>

May 10-12



European Conference on Rare Diseases and Orphan Products
Vienna, Austria
More information at <https://www.rare-diseases.eu/>

May 20-24



2018 World Federation of Hemophilia World Congress
Glasgow, Scotland
More information at <https://www.wfh.org/congress/en/home>

June 14-17



2018 European Hematology Association Annual Congress
Stockholm, Sweden
More information at <http://eha-2018.org/index.html>

July 18-21



Annual Scientific and Standardization Committee Meeting of the International Society on Thrombosis and Haemostasis (ISTH)
Dublin, Ireland
More information at <http://www.ssc2018.org/>



We would like to sincerely thank the whole community for all the support, time and positive energy that contributed to our work!

Our best wishes for a restful and joyful holiday season and a happy 2018!

The EHC Staff