EHC
european haemophilia consortium

EHC NEWSLETTER

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EHC Newsletter May 2017

Table of Contents

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President and CEO Report

It is not possible to think about where we want 2017 to take us without considering current state of affairs throughout Europe. From BREXIT to significant political elections in Europe and abroad, effects of these turbulent times on the health care environment are unavoidable. Preparedness is key and the European Haemophilia Consortium (EHC) has continuously evolved its work both to be prepared for, as well as to take leadership in, changes and new efforts to tackle inequities in haemophilia care across European countries.

Brian O'Mahony, EHC President

Amanda Bok, EHC CEO

This is the basis of our most ambitious area of work yet, which we proudly launched last month during World Haemophilia Day (see pg 14): our PARTNERS programme. Procurement of Affordable Replacement Therapies - Network of European Relevant Stakeholders, PARTNERS, seeks to substantially and significantly increase patients' access to factor replacement therapies in up to 14 eligible countries that meet defined EHC criteria – derived from the most recent haemophilia recommendations of the Wildbad Kreuth process and the Council of Europe's European Directorate for the Quality of Medicines and Healthcare (EDQM) – and do so in a manageable and sustainable way with no immediate change to national budgets and a quick yet affordable step-wise increase in national consumption to levels recommended by the EDQM/Wildbad Kreuth soon after entering the programme. Ambitious and innovative, PARTNERS – as the name implies – is only possible thanks to bold partnerships between multiple key stakeholders that include national Ministries of Health, national payers, national patient organisations, clinicians and industry. Particularly key is the participation of pharmaceutical companies, without which PARTNERS would not be possible, and we are delighted to announce that Kedrion Biopharma, Pfizer and Sobi are the first to have provided signed confirmation of their intent to support the development of this programme. Danish philosopher Søren Kierkegaard said: "the most painful state of being is remembering the future, particularly the one you'll never have." The real substantial change that PARTNERS offers is the possibility of multiple stakeholders at national and European levels working together to offer a future to people with haemophilia who in many countries still live in pain and in the shadows of their society. You can find more information about this program on pg 18, where we also introduce Declan Noone, who will lead the implementation of PARTNERS.

It was in a similar spirit of partnership that we chose last month to celebrate World Haemophilia Day with – and hosted by – EDQM in Strasbourg. Our event (see pg 14) brought together numerous patient and clinician voices from all across Europe to highlight just how important the 2016 Wildbad Kreuth IV recommendations are to our community (and we were pleased to see that paper published in Haemophilia shortly before the event – see). In addition to the event, the EHC is currently producing a short video about the 12 haemophilia recommendations to help disseminate them to as wide an audience as possible – stay tuned for an upcoming release.

Speaking of videos, we are delighted to continue our collaboration with Goran Kapetanovic, director/producer of the moving documentary 'Haemophilia Stories,' and to announce that a second and shorter edition, 'Inhibitor Stories', will be coming soon. Through personal accounts, this short film will highlight the disparities in access to inhibitor treatment across Europe and the very different

outcomes they lead to, while also underscoring the strength, diversity and commitment of our European inhibitor community and outlining some manageable national steps that could be taken to markedly improve the quality of life of people with haemophilia and inhibitors in countries with restricted access to treatment and care.

Community was also foremost on our minds last month during our fourth Youth Leadership workshop, held from 7-9 April in Amsterdam, which brought together a new group of 16 motivated and talented young leaders from 12 countries to talk about volunteerism, engagement and leadership in our European patient community context (see pg 12).

Finally, we are pleased to announce that our paper 'Haemophilia Care in Europe: Survey of 37 Countries' was last month accepted to be published in *Haemophilia* and will appear shortly. Meanwhile we have closed and are writing up our 2016 survey on hepatitis C as well as planning our 2017 survey, which will be rolled out later this year.

All news in this newsletter will not, however, be positive. It was with deep sadness and regret that we learned of the passing of two people last month that both played key roles in the history of the EHC and indeed in the global haemophilia patient community.

Gordon Clarke, from Northern Ireland, had haemophilia and numerous co-morbidities but that never stopped him from being strongly and actively involved both with the EHC – amongst other things as a past President – and the World Federation of Hemophilia (WFH), who awarded him a Lifetime Achievement Award in 2014. We knew Gordon well and we imagine that most readers of this newsletter probably did as well. In many ways he was larger than life; he was bold, he was brazen in the ways that make a difference to others, and he was passionately dedicated to our international patient community, to whom he directed most of his life's efforts and energies (see more on pg 22).

Edmond Secq, from Belgium, was a person with haemophilia and a driving force in the EHC, amongst other things in moving the organisation to Brussels as well as drawing up the EHC statutes together with then-President Hubert Hartl from Austria. Sadly, not all of us knew Edmond personally, but we are grateful for his contributions to the EHC's organisational building blocks and for his personal efforts towards building a strong and impactful patient community in Europe (see more on pg 23).

Gordon and Edmond both left their personal and unique marks on our organisation and more importantly on our lives. Both men will be sorely missed. We extend our most heartfelt condolences to their families and loved ones, and we remember them, their spirits and their life's work. They succeeded in helping to improve treatment and care for people with haemophilia everywhere – and we will continue their life's work.

EHC NEWS

Comprehensive care in haemophilia: Inhibitor Management

Interview with Professor Johannes Oldenburg, taken by Raia Mihaylova, EHC Communications Officer

Today, people with haemophilia (PWH) have a better quality of life than ever before. Receiving news of inhibitor development, however, can feel as if being thrown back into what the situation was like a decade ago - expensive and limited treatment options, lack of information and further lifestyle restrictions.

Doctors are still struggling to identify the reason why some PWH form inhibitors and others don't, making it the most serious challenge in haemophilia treatment today. After a period of no significant progress, better understanding of some inhibitor aspects in recent years seems to be shifting us closer to the answers. Scientists are looking more and more into whether there is an increased chance of developing an inhibitor depending on the haemophilia treatment used - plasma-derived coagulation factor concentrate or recombinant clotting factor product. New therapies currently in clinical trials show promising results in reducing the number of bleeds and doctors remain optimistic that intensive research efforts will eventually lead us to treating inhibitors more effectively or to preventing them altogether.

I met with Professor Johannes Oldenburg to discuss in greater detail some of the questions around inhibitor development. He is a professor and the director of the Institute of Experimental Haematology and Transfusion Medicine and the Haemophilia Centre at the University Clinic in Bonn, Germany.

Professor Oldenburg speaking at the EHC Round Table in March (See pg 8)

Inhibitors develop in 25-30 per cent of patients with severe haemophilia A and in 1-5 per cent of those with severe haemophilia B. Why is it more common in the first group?

The driving force for inhibitor formation are so called null mutations, severe mutations, that do not make the endogenous protein in patients. About 85 per cent of all severe haemophilia A patients carry such a null mutation, while in haemophilia B, it's only 20 per cent of the people. So, this different mutation spectrum is the reason why.

Is there a difference in the approach of treating inhibitors in patients with haemophilia A and those with haemophilia B?

In principle, treatment for both is the same – receiving immune tolerance therapy (ITI). However, because of longer half-life products, most haemophilia B patients are getting their clotting factor concentrates twice a week, while for haemophilia A, the most preferred regiment is three times a week. And then there is the challenge that about half of patients with haemophilia B and inhibitors develop an anaphylactic reaction to infused factor IX. We then, of course, have to come up with a different approach.

What makes you suspect someone has inhibitors?

The risk of developing an inhibitor is mainly within the first 50 exposure days after receiving treatment. The median is between 10-14 exposure days. The clinical presentation can be that patients have a bleed and do not respond to their regular dose of clotting factor concentrate. Or it is a laboratory finding through a screening test used to detect inhibitors. Nowadays, in many centres, since we know the risk time of developing inhibitor is the beginning, we ask patients to come back for observation every 3 to 5 factor replacements, so that we can make an early diagnosis.

There are also certain factors that we keep an eye out for. This could be inflammatory conditions, such as undergoing surgery, or looking at the intensity of haemophilia treatment. The higher the dose of treatment and its more frequent administration means a higher risk of inhibitor formation.

How do you formulate treatment for each patient, is it individualised?

In Germany, we are lucky because we can provide every patient who develops an inhibitor with an inhibitor eradication protocol. As soon as we detect it, we speak with the family about the possible options of eliminating the inhibitor. The patient is then hospitalized and we try to start treatment right away. If it's a high inhibitor titre patient with more than 5 Bethesda units, then we start with a high dose protocol that is between 100-200 units every day. In Germany, we even give the clotting factor concentrate for inhibitor elimination every 12 hours, so it's a very intensive treatment. If it's a low inhibitor titre, the patients usually receive classical prophylaxis and we observe how the inhibitor is developing, if it's becoming stronger. These two are the main protocols – the high dose protocol for high titre inhibitors and the low dose protocol, very comparable to a standard prophylaxis, for low titre inhibitors.

What is the success rate of getting rid of inhibitors?

In high titre inhibitors, all published data so far show about a 70 per cent success rate of getting rid of the inhibitor. This means that in about 2/3 of the patients, you can eliminate the inhibitor, or you can eradicate it to an extent where the patient can receive standard clotting factor concentrate.

What are your views on the Survey of Inhibitors in Plasma-Product Exposed Toddlers (SIPPET) study? *(This study is the first randomised controlled trial to ever be conducted in haemophilia and to look at whether the risk of developing inhibitors is higher from using plasma-derived factor concentrate or recombinant factor.)*

The SIPPET study is great because it's the first study that really randomised patients. It also confirms the feelings that many healthcare providers had before, which is that plasma-derived products may lead to lower inhibitor incidents. I personally think that this data is solid. We still don't have enough information to the extent where countries can direct policies towards which treatment to use at the start of therapy. However, there are already some reasonable arguments to use plasma-derived concentrate in previously untreated patients (PUPs).

Can you tell me a little more about what the situation is like in your country (Germany) when it comes to inhibitor treatment?

In Germany we provide free treatment to inhibitor patients, which includes attempts for the eradication of inhibitors, but also the prophylactic application of by-passing agents. Both are covered by health insurance. We have to communicate with and inform the insurance companies of our treatment plan, but we don't get any rejections.

Also, the main and most frequently used protocol for tolerisation, the Bonn protocol*, was developed in Germany, so immune tolerance therapy has a long tradition in Germany.

**The Bonn Protocol was established in Bonn in the late 1970's. It involves regular administration of factor replacement over a prolonged period of time in conjunction with the use of activated prothrombin complex concentrate. Dosing rates vary and patients using this method traditionally stay on it for several months to several years.*

Will treatment eventually become more affordable/accessible?

My expectation is that more immunosuppressive drugs will be included in treatment plans. They will facilitate inhibitor eradication by making it achievable with less amount of clotting factor concentrations, so that is one way the cost may come down. In general, the cost of clotting factor concentrates is lowering, especially in those countries where tender programs are applied. And then there are the new drugs that can be used for prophylaxis in inhibitor patients, they may completely change the situation because they will provide constant bleed protection and in principle, could be much cheaper than clotting factor concentrates.

So, we can expect significant change in the next 5-10 years?

Yes, we will see a complete change, even in the so-called "developing" countries. There is a light on the horizon with new, non-substitutive therapies, which can be used for prophylactic treatment in inhibitor patients. There are a number of new molecules that are being developed, which can be just as efficient as the treatment for patients that don't have inhibitors. The first of these products will be licenced in the second half of 2018, so it is not far away!

EHC Round Table on Clinical Trials in Haemophilia A

By Raia Mihaylova, EHC Communications Officer

There is an overall excitement throughout the haemophilia community about the future. Constant improvements in treatment are paving the way for a number of novel therapies to emerge, with the hope that they will target areas where current methods have failed. Their development, however, is just the first step in a long process of testing, observation and assessment. Before they are licensed for patients' use, it is of crucial importance to generate enough data on their safety and efficacy.

This is when clinical trials enter the equation. It is thanks to the results of these carefully planned and conducted studies that regulators are able to determine if a medicinal product can be released on the market. Many are currently underway for newly developed haemophilia treatments.

For this reason on March 7th, the European Haemophilia Consortium (EHC) held its first Round Table of Stakeholders on the topic of 'Clinical Trials in Haemophilia.' More than 50 patients, healthcare professionals and representatives from the pharmaceutical industry attended the meeting at the European Parliament in Brussels and contributed to discussions on epidemiological data, gene therapy, inhibitor formation and more.

As such issues tend to be very technical, it is important to us that we use the Round Table as a platform from which their complexity is broken down into easily understandable language. Our four prominent speakers – Professor Frits Rosendaal, Professor Johannes Oldenburg, Professor Pier Mannuccio Mannucci and Professor Flora Peyvandi – did so with detailed presentations that covered all aspects of clinical trials and the questions that surround them.

EHC President Brian O'Mahony, Professor Flora Peyvandi, Professor Frits Rosendaal, MEP Norica Nicolai, Professor Mannuccio Mannucci and Professor Johannes Oldernburg

Participants were first welcomed by Member of the European Parliament Mrs Norica Nicolai (Romania/ALDE), who hosted the event, and by MEP Dr Miroslav Mikolášik (Slovakia/EPP). Both are long-time supporters of EHCs' efforts to improve the quality of life of haemophilia patients and help to bring political attention to patients' concerns.

In her opening speech, Mrs Nicolai shared how she was introduced to the topic of haemophilia and how impressed she was with the community's efforts in battling for improvements.

Professor Flora Peyvandi then led the conversation towards the scientific part of the programme:

"As haemophilia and other clotting disorders are rare, the number of patients, fortunately, is low, but this makes it much more difficult to have a good clinical trial with well-established results. The reason

we thought with the EHC that it is time to get together with patient organisations and have the pharmaceutical representatives in the room, is so that we can understand together whether what we have been doing as a method for data collection is reflective of the real situation or if we need to change our strategy."

Professor Peyvandi chaired the event

Professor Frits Rosendaal was first to present, starting with an introduction to the collection of epidemiological data. The optimal data collection method depends on the content, aspect of the disease, methodology and the type of questions that are attempted to be answered. One major distinction is whether researchers are looking into evaluating the effects of a drug – does it do what it is supposed to do – or if they are observing its side effects. Consensus tells us that drug evaluation and comparison requires a randomised trial.

"The randomised controlled trial is really different from observational studies because it does away with all the bias of groups being different, which makes it by far the most powerful design. That's the reason that studies on the effect of drugs have to be randomised, so you can take away the confounding element," explained Professor Rosendaal.

On the other hand, if you are looking at side effects, observational studies are usually sufficient in obtaining information. Side effects are unexpected and their risk factors are often unknown, so the tested groups are initially looked at as having no differences between them.

When it comes to clinical trials specifically for haemophilia treatment, the optimal design has to take into account the disorders' low prevalence. Challenges, such as difficult recruitment of patients and lack of funding, often limit trial size and frequency. There is no doubt, however, that studies for side effects need to be independent, transparent and open to investigators. If they are observational, they should include all patients in a region early on and unselectively. Data collection should be harmonised through registers that allow cohort and case-control analysis. Still, randomised trials should be attempted when necessary and possible.

Professor Rosendaal presented on "Collection of epidemiological data in haemophilia."

Through examples, Professor Rosendaal also clarified the difference between evaluating the harm and benefit of a drug. To prove that one drug is better than another, people would want to see very solid evidence. For harm, the threshold is much lower – it doesn't need to be proven, suspicion is usually enough.

Taking the floor next was Professor Johannes Oldenburg from the University of Bonn, who focused on "Long-term observation of efficacy for novel therapies in haemophilia." He set the scene by explaining how the current birth of new products will most likely change the paradigm of the current birth of new

products will most likely change the paradigm of the current haemophilia treatment. One new class of products is the classical substitution clotting factors with extended half-life. For haemophilia A, these products have a half-life extension from 12 hours to about 18 hours. Independent of which technology is used to extend the half-life, at 18 hours, they are all limited because factor VIII is bound to the von Willebrand factor and is cleared by it. This means that currently, the number of injections can be reduced from three to two times per week.

Professor Oldenburg explained what improvements we can expect from new treatment products

For haemophilia B, the difference is much more significant. The half-life extension goes from 20 hours to about 100 hours, giving it a five-fold increase and reducing injections from twice a week down to once every two weeks.

For new non-substitutive therapies, there are mainly three technologies – anti TFPI, Si-RNA downregulation of antithrombin and bi-specific antibody. These therapies can all be given subcutaneously and they no longer need IV application. As they have a long half-life - up to 4.5 weeks - they can be given weekly, or even monthly. With their broad spectrum, they can be applied to patients with inhibitors and to patients without.

Professor Oldenburg then spoke about the long-term efficacy observation of these novel therapies. Expected outcomes include, amongst other things, lower annual bleed rates, fewer joint bleeds, reduced number of injections and improved adherence. When monitoring patients to learn about the efficacy, the main outcome parameter is joint arthropathy. As he explained, the degree of joint arthropathy is something like a cumulative memory of the quality of the treatment that has been developed over decades in the background of a prophylactic regiment. The aim is, of course, to avoid bleeds and to avoid the development of joint arthropathy by starting treatment early.

Participants had the chance to ask questions and were heavily involved in discussions throughout the event

Professor Peyvandi built on to the presentation by speaking on “Safety of novel therapies.” She began with the question of how and to whom adverse events should be reported and illustrated the answer by explaining the current regulatory process and the downfalls of pre-marketing trials. The availability of a large number of new treatment products calls for a systematic post-marketing surveillance. To really be able to collect data on safety, all haemophilia patients, regardless of severity and age, who use a new drug for the first time should be registered.

As we are aware, the crucial question in haemophilia is what causes inhibitors to treatment. For emerging novel therapies, the question becomes whether they will also cause inhibitors and whether

Professor Mannucci focused on previously untreated patients (PUPs) in clinical trials

they can be used in people with inhibitors in the first place. Professor Pier-Mannuccio Mannucci from the University of Milan tackled those and other questions in the last presentation of the day – "PUPs in clinical trials." He first briefly summarized the incidents of inhibitors development rate in previously untreated patients (PUPs) and previously treated patients (PTPs). He then dealt with the chronology of the European Medicines Agency (EMA) regulatory guidelines for the marketing authorization of antihemophilic medicines, emphasizing that in the beginning they didn't require enrolment of any PUPs in clinical trials in order to license new FVIII products, whereas now they do. There is still no set consensus on whether it's PUPs or PTPs that are the optimal clinical model to evaluate the immunogenicity of new FVIII products. But based on recommendations by the International Society on Thrombosis and Haemostasis (ISTH), studies to analyse the immunogenicity of new products should be performed in PTPs with more than 150 exposure days, while PUPs should be reserved for studies on the natural history of inhibitor development or for studies of new concentrates for which viral safety is an issue. To conclude, Professor Mannucci shared that the Committee for Medicinal Products for Human Use (CHMP) of the EMA has considered revising the current clinical guidelines on FVIII, in regards to the requirement of PUPs in clinical trials and establishment of a minimum core parameter set on data collection in haemophilia registries for addressing inhibitor detection.

All four presentations were followed with great interest and prompted in-depth discussions with the participants at the end of the event.

Our next Round Table will be held on June 27th on the topic of "Orthopaedic Aspects in Haemophilia." We invite you to join us for a comprehensive coverage of the topic.

Tools to pursue dreams: Youth Leadership Workshop

By Raia Mihaylova, EHC Communications Officer

"Build your leaders and you will have a vibrant community that will accomplish change."

From April 7th- 9th, the European Haemophilia Consortium (EHC) held its fourth Youth Leadership Workshop in the vibrant city of Amsterdam. Under the now-traditional title of "*Speak Up, Stand Out, Join the Conversation,"* this years' workshop was designed to equip participants with strategies on how to be more actively involved in their National Member Organisations.

Sixteen participants between the age of 21-30 were welcomed and brought the diversity of Austria, Estonia, Finland, France, Germany, Ireland, the Netherlands, Portugal, Romania, Serbia, Slovakia and Switzerland together. Their energy and enthusiasm for learning how to join their local organisations in moving forward made it obvious that the future of haemophilia is in good hands. For three days, they were involved in activities that combined presentations from experienced trainers with practical and interactive exercises that consolidated the skills they had learned. The themes of the workshop focused on how to engage and retain volunteers, internal and external communication, including engagement with the pharmaceutical industry, and strategic planning and managing of a project.

Outside the "classroom," real-life experience took over as friendships were easily formed and participants talked freely about every-day experiences and challenges.

The weekend was quick to end but a lot was gained. We are excited to welcome a new class every year, but just as important is retaining the bond we formed with each previous group. Participants are encouraged to continue their involvement and attend other EHC events, such as our Leadership Conference and EHC Conference, and some have become members of various EHC committees. Such is the story of Naja Skouw-Rasmussen (see following article), who took part in the first Youth Leadership workshop three years ago and is now part of EHC's Steering Committee.

The Youth Leadership workshop was made possible by funding from Novo Nordisk, Roche and Shire, whom we thank for their support.

From youth to senior: not a matter of age

A reflection on the move from being a participant at the EHC Youth Leadership workshop to now being a member of the EHC Steering Committee.

By Naja Skouw-Rasmussen, EHC Steering Committee member

Back in 2014, I was selected to participate at the very first European Haemophilia Consortium (EHC) Youth Leadership workshop in Amsterdam. It was the first time the EHC had hosted an empowerment workshop for youth. I was really looking forward to being a part of this then-new initiative and my expectations were met. Even though it is three years ago now, one of the things I remember best was that the youth workshop gave me an opportunity to discuss the various aspects of being young and an active member of our respective countries' bleeding disorder community. It was also an opportunity to learn more about how different countries are organised, which forced us to reflect upon the pro's and con's of the way things were organised in our own country. And yes, it was also an opportunity for us, who participated, to get to know each other, which makes it so much easier to work together across countries today.

Naja as a participant in the first EHC Youth Leadership workshop

Of course, I was not completely "green" in terms of being engaged in the bleeding disorder community at an organisational level. At that time, I had already been a member of the Danish Haemophilia Society's board for some years. But it is very healthy to have time to learn, discuss and reflect about your own role and engagement in the organisation. And I think that I gained more from the workshop because I could put it into a context straight away. Overall, the EHC Youth Leadership workshop created a space for the participants to learn, to ask many question and explore solutions in a collaborative way.

One fun fact is that when I was a participant, we were a small group discussing the prospect of establishing a European network for women affected with a bleeding disorder. Today, everyone from this small group has been a part of establishing the EHC Working Group on Women and Bleeding Disorders.

The title 'From youth to senior' is not really a reference to getting older in age, but merely a reference to the change from getting to know the in's and out's of my Society to taking an active part in the decision-making and ensuring the development of our Society and committee.

World Haemophilia Day 2017

By Raia Mihaylova, EHC Communications Officer

World Haemophilia Day was first marked in 1989. It was started by the World Federation of Hemophilia (WFH) to increase global awareness of the condition; April 17th was selected in honour of its founders' birthday, Frank Schnabel.

The European Haemophilia Consortium (EHC) has used the day not only as an opportunity to bring attention to challenges faced by the haemophilia community, but also as a chance to come up with concrete solutions.

This year was especially exciting for us. On April 19th, we marked World Haemophilia Day by highlighting the latest Wildbad Kreuth recommendations (see pg 15) on optimal treatment of haemophilia in Europe and by unveiling important details around our PARTNERS programme (see pg 18). Both concepts have the ability to lead to real, substantial change for the long run.

The event was hosted by the European Directorate for the Quality of Medicines and Healthcare (EDQM), part of the Council of Europe, in Strasbourg, France. Over 50 patients, healthcare professionals, regulators and pharmaceutical industry representatives joined us for discussions on the importance of the consensus recommendations and what steps should be taken to implement them in countries across Europe.

The recommendations are the outcome of the latest European symposium 'Wildbad Kreuth IV' Initiative on the optimal use of clotting factors and platelets, held in May 2016 and organised by the University of Munich, the Paul-Ehrlich-Institute and the EDQM. During these series of meetings, experts from 36 countries issued consensus recommendations on how to address current challenges in haemophilia treatment and care.

Professor Wolfgang Schramm, one of the speakers, began with an overview of the consensus recommendations

Besides tackling issues in haemophilia treatment, these recommendations are also of incredible importance in that they give our National Member Organisations the tools to allow them to advocate for maintaining or improving haemophilia care in their countries.

Each of the invited speakers used their presentation to highlight one of the twelve recommendations. Through sharing their personal stories, it was evident that these recommendations are not just words in a document, they are very real concepts that impact patients' quality of life and future.

During the event, the EHC also unveiled a new programme aimed at improving access to treatment for people with bleeding disorders in Europe. The Procurement of Affordable Replacement Therapies – Network of European Relevant Stakeholders (PARTNERS) programme seeks to ensure increased and sustainable access to treatment in up to fourteen European countries that do not meet the minimum standards of haemophilia care, as outlined by the 2016 consensus recommendations. These countries, where level of factor VIII and IX consumption is below 4IU/capita and .5IU/capita, respectively, provide little or no prophylactic treatment for children and adults with severe haemophilia.

The highlight of the event was the signing of a Consensus Agreement between the EHC and Kedrion Biopharma, Pfizer and Sobi. This confirmed the pharmaceutical companies' intent to support the development of the PARTNERS programme, without which PARTNERS would not be possible.

The day concluded with insightful discussions and an overall feeling that we're on the way to a better future for haemophilia and other rare bleeding disorder patients!

Signing of the Consensus Agreement

Speakers, EHC Staff and members of the EHC Steering Committee and the Medical Advisory Group

Wildbad Kreuth Consensus 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.)

First European Inhibitor Summit

Barretstown, Ireland

From 1-4 December 2016, the European Haemophilia Consortium (EHC) held its first Inhibitor Summit in Barretstown, Ireland. The event was organised to bring together people with haemophilia and inhibitors to meet and learn in detail how to best manage the condition. More than 90 participants from 27 European countries attended. The programme was a mixture of lectures on scientific and clinical topics such as inhibitor formation and treatment, physiotherapy and pain management, as well as peer-to-peer sessions to share common experiences. Children took part in many leisure activities organised by the volunteers of Barretstown.

Experience of a parent

By Camilla Wensing, member of the Schweizerische Hämophilie-Gesellschaft, EHC Swiss NMO

My name is Camilla Wensing and I live in Interlaken, Switzerland. My son Enea has severe haemophilia B with inhibitors and an allergic reaction to FIX. We were invited to attend the first European Inhibitor Summit organised by the EHC, which took place in Barretstown, Ireland. The Barretstown camp was founded by Paul Newman, where in his words, kids could "raise a little hell."

It is very challenging to have a child with haemophilia and inhibitors, and the Summit provided an important opportunity for us to meet with families from all over Europe facing similar challenges, to share our experiences and learn from one other, which I believe provides vital support for patients and their caregivers. It was also sobering to discover that not all countries in Europe offer patients the same standard of care.

I very was impressed by the excellent organisation of this large event, as well as by the helpfulness of the EHC and Barretstown staff. I would also like to thank the many healthcare professionals who donated their knowledge and time to inform us about the latest medical information and treatment updates.

All in all, Enea and I thoroughly enjoyed ourselves during the Summit, and I cannot emphasize enough how inspiring and fun it was to meet the other participants. The four days simply flew by and it all ended far too soon!

What impressed me the most?

- The joy and determination of the other participants, both caregivers and patients.
- The interesting medical updates, the good discussions in the smaller workshops, and the chance to get to know the other participants better. Unfortunately, the size of the event meant that there was not enough time to get to know everyone.

I am already looking forward to the Inhibitor Summit of 2017! Many thanks to the EHC for making it happen!!!

The next Inhibitor Summit will be held from 28 November to 3rd December 2017. People with inhibitors and their families who wish to attend this event should get in touch with their national patient organisation for nomination. The Inhibitor Summit, part of EHC's European Inhibitor Network (EIN), was made possible by funding from Shire; we are grateful for their support.

New Year, New Us - EHC welcomes new staff member

By Laura Savini, EHC Public Policy Officer

In this issue, we are delighted to introduce the newest team member of the European Haemophilia Consortium (EHC), Mr Declan Noone, who joined us in January as PARTNERS project consultant.

Declan has been involved in the haemophilia community both in Ireland, his home country, and at international level for many years. Some of our more regular readers may recall that he was chair of the EHC Data and Economics Committee and many others will probably know him through his work with the Irish Haemophilia Society (IHS) and the World Federation of Hemophilia (WFH). However, as it is a tradition of the EHC Newsletter, we have decided to give him a proper introduction through this 'meet the EHC staff' article.

Please introduce yourself:

I have haemophilia A severe and as a result I was involved early on in the activities of the IHS. When I turned 18, I started to volunteer in the IHS' children's programme. As with many other volunteers in the haemophilia community, I was asked if I wanted to attend a WFH Global National Member Organisation (NMO) training in Bangkok, Thailand, in 2006, which of course I accepted and this is how I subsequently became active on the international level and have remained there ever since.

Declan speaking about PARTNERS during a meeting in Paris in February

During this training, I understood the importance of becoming more involved in the IHS activities and when I came back, I decided to join the IHS board for two years, after which I was offered a job as IHS staff member. In this position, I first worked on the children's programme but moved quickly to work on access to treatment (including access to hepatitis C (HCV) treatment), which then really became the focus of my work at the IHS. This included activities such as the setting-up and becoming involved in the Irish tender board. For this purpose, I conducted several activities such as horizon scanning, i.e. looking at what products were under development and had a potential to reach the European / international market; looking at which products were available in other countries and how they were purchased, etc. With regard to HCV treatment, I created a database on products available and produced detailed information, though a newsletter, on medical and scientific advances for HCV treatment.

Last year, I took a sabbatical and in September 2016 I enrolled in a Master's programme in health economics, which I am currently finishing. When the job advertisement for PARTNERS project consultant came up, it seemed like a natural fit to my profile and I just thought: 'Go for it!' I am glad I did and I am really excited about this project.

Can you briefly introduce the PARTNERS programme and tell me one thing that excites you about it?

There are lots of exciting things about this project. First of all, it is an attempt to increase access to treatment for patients in countries with very little product and it will be done without increasing costs for the government in the short-term. For countries to join, they need to not comply with treatment standards set by the Council of Europe resolution CM(2015)3 and thereby not meeting certain standards such as minimum use of FVIII or providing prophylaxis to children. The key aspect of this project is that we want to persuade governments to hold national tenders to increase their purchasing power. This would be achieved by purchasing larger quantities anywhere under a maximum price for which they are willing to purchase product. This would allow companies interested in bidding to offer

products for up to a maximum ceiling price. Our hope is that some countries may also decide to partner up and do a joint purchase. This would be particularly beneficial for countries with only a couple of million people in their population. However, the real benefit of this project will be to help countries to plan ahead and streamline their purchasing process hence ensuring that adequate budgets are forecasted and offering some predictability to companies in terms of letting them know the amounts they intend to purchase. From experience the more organised and predictable a process is, the cheaper it gets because it ensures that no money is wasted but also that the system is tailored to your country's needs.

For instance, we see that in some countries prices are much higher than in others. However, when you break it down, you note that this is due to some system's inefficiencies, such as the way products are purchased or distributed. If you can make the system more direct then you can cut a lot of the superfluous costs.

The positive thing with haemophilia is that we are a 'small' patient population, which can be easily managed in a centralised way, hence maximising the use of the budget allocated for haemophilia care. This is another part of the job that I am particularly excited about. PARTNERS is not just about purchasing products, although it is a significant component of this project; it is also about advising governments on how they can best re-design their haemophilia care so that it is efficient and maximizes all available resources. A key element to achieve this is to include in an active and meaningful way both clinicians and patients as they are truly the experts of their healthcare system and can provide an insider look on things that work and things that can be improved. This point is also true for PARTNERS, as the name indicates: we cannot go into a country and try to apply a system that works in another country. Instead we need to understand the country's system – and to do so include patients and clinicians – so that we can work out what would be the best way for them to organise their haemophilia care. I think that two elements that we will try to promote heavily are home treatment and prophylaxis because we know that in the long-term both of those things are not only hugely beneficial for the patient, but they also save the healthcare system a lot of money. In fact, they avoid frequent and costly hospital visits.

What are three hobbies you have?

In my free time, I love rock climbing, I swim and I play a little chess. However, above all, and in true Irish fashion, I am a huge rugby fan! I have always wanted to play rugby because that's pretty much what every child in Ireland wants to do. I recently bought a house near a rugby field so it seems that I cannot quite get away from it.

What is one thing that might surprise people about you?

I paid my college fees by juggling fire! When I was younger, I used to be into something called poi or fire staff. In short, you have a six-foot (or two metre) fire staff, you fire it at both ends and use your hands and body to twirl it around. Once or twice a month I would go to shopping centres, bask and get paid for it. That is basically how I was able to pay for my college tuition fees.

When can our members meet you?

I will be speaking and moderating the EHC Workshop on Tenders and Procurement in September in Sofia, Bulgaria. For countries taking part in PARTNERS, I will be visiting them throughout the year.

Thank you, Declan, for this introduction. We are delighted to have you onboard and wish you much success with PARTNERS.

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

EHC forms a new working group on women in the bleeding disorders community

By Evelyn Grimberg, volunteer at the Nederlandse Vereniging van Hemofilie-Patiënten (NVPH), EHC Dutch NMO, and member of EHC's Working Group on Women and Bleeding Disorders.

During the European Haemophilia Consortium (EHC) Conference in Stavanger in October 2016, women and men affected by bleeding disorders were invited to attend an informal meeting about women in the bleeding disorder community. The aim of the meeting was to bring together representatives from EHC National Member Organisations (NMO) in Europe with an interest in women's activities and to exchange information on national activities organised for women with bleeding disorders, carriers and women sharing their life with someone with a bleeding disorder. It was the next step following the first workshop in Belgrade in 2015, "Women in the bleeding disorders community: starting a European conversation."

After a short presentation at the 2016 meeting on "Why is it so important to work with and for women?" and examples of what could be done in each country, the participants put in place a proposal to create a group that would be a contact point and a creator of activities for women in Europe. The purpose of the group is to create awareness, recognition, support and education about and for women in the bleeding disorder community. This includes women with bleeding disorders, carriers and partners of people with rare bleeding disorders (haemophilia, von Willebrand Disease, platelets disorders, etc.). The group is currently composed of Naja Skouw-Rasmussen - Denmark, Yannick Collé - France, Aleksandra Ilijin - Serbia, Christina Burgess - United Kingdom, Marija Nakeska - Macedonia, Marie Lahn Rømcke - Norway and Evelyn Grimberg - Netherlands.

The group has proposed an activity plan for the coming years, which was accepted by the EHC Steering Committee in February, and includes:

1. Communication and awareness
    - *Information about carriers with symptoms of bleeding disorders; information about the quality of life of carriers and women with bleeding disorders; education of doctors; recognition; newly diagnosed families; education within and outside of NMOs*
2. Medical questions
    - *Gynaecological issues; diagnoses of gynaecological issues; treatment options; puberty (menorrhagia); prenatal diagnosis; dietary information; pregnancy and childbirth*
3. Psychological and social well-being
    - *Emotional support; family planning; sharing of experiences; relationship with partner; equal support; male-female interaction; quality of life issues; acceptance by family and support.*

This coming year, the group is going to focus on seeking information from all EHC NMOs in order to build a European network. Each NMO should have received an email asking about the different womens' activities in their organisation. We are also asking for contact details of a person in the NMO who would be keen to help improve the situation for women in the bleeding disorder community. With these contact details, we can be in touch about work relating to women and we can also inform NMOs about activities coming up. We want to make the NMOs aware of the issues experienced by women with bleeding disorders by providing information on different topics on the EHC website, through the EHC newsletter, and by being visible and raising our voice at meetings organised by the EHC.

As can be seen, there is a lot of motivation and much to do. The group is more than eager and happy to start this work. We have many plans for the coming years, and invite the community to take part in them!

If you have any questions or ideas, please contact us through the EHC or through the contact form on the website.

IN LOVING MEMORY

Gordon Clarke – An Appreciation by Brian O'Mahony, EHC President

Gordon Clarke

Gordon Clarke, who sadly passed away last week, had a long and varied involvement at a high level as a volunteer leader in the haemophilia community. Gordon was from Northern Ireland and had a deep involvement in the United Kingdom haemophilia society. His professional expertise and knowledge as a hospital administrator were very beneficial to the haemophilia community. At the time when the United Kingdom held the secretariat of the European Haemophilia Consortium (EHC), Gordon also served as Chairman of the EHC for a three-year period from September 1999 to May 2003. Gordon additionally served on the Executive Committee of the World Federation of Hemophilia (WFH) from 2002 to 2010. He was responsible for the WFH Monographs on Governance and Action Planning and participated and facilitated many training sessions for the WFH. He also served for a time as Vice President for programmes and oversaw the WFH's advocacy programme while it ran. In 2014, he received a Lifetime Achievement Award from the WFH. In later years, he was very proud to be a destination ambassador for Northern Ireland and was delighted that the EHC Conference took place in his beloved Belfast in 2014. I had the pleasure and privilege of working with Gordon when I was WFH President and Gordon was on the Board. We also worked together at the EHC. He had a warm and engaging personality, quick to laugh and always prepared with a – usually long and complex – joke to make a long meeting seem a little shorter. I first met Gordon at the WFH Congress in Brazil in 1984 – a first Congress for both of us. I recall drawing up his factor concentrate for his infusion while he injected his insulin for his diabetes. Not a minute was to be lost as we were meeting several other young men with haemophilia for dinner. I have quite a few happy memories of working and spending time with Gordon. His skill and commitment to the community were always evident but it was his humour, humanity and empathy which I will remember with particular fondness. He loved fishing. He was devoted to his wife Jacqui and his children and grandchildren. His commitment over so many years to the haemophilia community means he will be remembered fondly, not only by his immediate family, but by all of us in his wider and global haemophilia family.

Those who would like to express their condolences can do so here.

IN LOVING MEMORY

Edmond Secq – An Appreciation by Jo Eerens, EHC Membership Officer

Mr Edmond Secq with Hubert Hartl, November 2006

On 24 of April 2017, Mr Edmond Secq passed away in Brussels at the age of 89 years. Edmond Secq was a haemophilia patient who joined the Belgian Haemophilia Society in 1969 as its secretary and stayed in that role until 2010. Through his passion for European affairs, he was one of the driving forces behind the European Haemophilia Consortium (EHC). Together with his spouse, Gusta Van Eepoel, he represented many times the Belgian Society at events of the World Federation of Hemophilia (WFH) and the EHC. For a long time, he was responsible for the finances of the EHC. Together with Hubert Hartl, he wrote and edited the statues of the EHC. Once the EHC was officially established in 2007, he seeded his mandate as EHC Steering Committee member and dedicated his life more and more to Gusta, who was dealing with difficult health issues.

We thank him for his precious contribution and dedication to the EHC and present to his family our deepest condolences. Those who would like can write a message on here, or write a posted message to the following address: Mr.and Mrs Terlinck-Secq, Bremlaan 33 , BE-3090 Overijse.

Meet Guus Wijfjes, the new president of the Dutch NMO

By Annebelle Nooteboom, translated by Erik Driessens, published in Faktor, winter 2016

'I know the issues at hand and understand the language'

Guus (55) was diagnosed with haemophilia A when he was eight years old. "I had two molars pulled out and I just kept on bleeding. Together with my parents and the dentist, we started looking for the cause,' he remembers. Upon finding out the reason, the family joined the Nederlandse Vereniging van Hemofilie-Patiënten (NVHP, Dutch National Member Organisation of the European Haemophilia Consortium (EHC)) and began their involvement in the haemophilia community.

Guus Wijffjes

Guus shares that his mother was quite shocked when hearing the diagnosis, because she was the carrier. At the insistence of both parents, the whole family eventually got tested. The results showed that all three of his sisters were also carriers and that two of his cousins have mild haemophilia. His daughter Rosine is a carrier too.

Unfortunately, Guus's mother passed away relatively young. "My three sisters told me, 'If mama could hear now that you are president of the society, she would be very proud'."

Medical background

Guus had an interest in the medical sector from a young age, which was the reason he chose an education that would allow him to become a nurse. He began his career as such at the Vrije Universiteit (VU) University Medical Centre and eventually worked in the intensive care unit of the Onze Lieve Vrouwe Gasthuis (OLVG) hospital in Amsterdam until he and his wife moved to Amersfoort. There, he became a team leader at the Meander Medical Centre. He spent eight years as senior nurse at the surgery ward and later became a care unit manager at the same ward.

'Doors will open for you'

After a few other intermediate positions, Guus is now also the Head of the Medicine Department at the Sint Antonius Hospital in Nieuwegein, a city in the Dutch province of Utrecht. He divides his time between work and involvement in the NVHP, where he had been Vice Chairman for 18 months before becoming President. It's a heavy work load, but as he says, 'You get a lot in return, doors will open for you and you can expand your network.'

Ambitious family

Together with his wife, Karin, Guus has two children, Ruben and Rosine, whom he talks about with great enthusiasm - Ruben has a job in the Navy and Rosine works with autistic young adults. His wife, Karin, works at the neonatal intensive care unit of the Wilhelmina Children's Hospital in Utrecht but the two met while she was at the children's ward of the VU University Medical Centre. "The children at the ward knew something was going on between us before we did," Guus says laughing.

'I like humour'

Guus has a positive attitude towards life and loves humour, because "laughter gives energy and relaxation." For recreation, he likes to go to concerts, and enjoys walking and gardening. He is a fanatical Feyenoord (Dutch professional football club) fan, and together with Karin, they like to go to the Veerse Meer (lake Veere), near the place where she was born.

We wish Guus much luck, success and rewarding experiences in his new position and we look forward to working together!

HemoNED, the Dutch haemophilia patient registry and digital infusion logbook – a work in progress!

By Mariette Driessens, board member of Medical Affairs, Nederlandse Vereniging van Hemofilie-Patiënten (NVHP), EHC Dutch NMO

Patient registries, particularly for rare diseases, are a powerful tool for observing all aspects of a disorder: which treatment works, who develops side effects and why, frequency of symptom occurrences and more. They help answer vital questions, which in turn are used to identify problems and solutions in treatment regimes.

The Beginning

The Dutch Haemophilia Treaters Society (NVHB), together with the Netherlands Haemophilia Patient Society - *Nederlandse Vereniging van Hemofilie-Patiënten* (NVHP) - applied for and received a two-year grant from the Netherlands Organisation for Health Research and Development (ZonMw) to develop a nationwide patient registry for people with haemophilia and other bleeding disorders, along with a digital infusion log (app) for patients. The project – named HemoNED – started in December 2015 and is led by project coordinator Dr Geertje Goedhart.

To assure its long-term continuation, additional funding for the registry is being provided by some pharmaceutical companies, making this a public-private partnership. In October 2016, the HemoNED Foundation was established to manage the registry; the board consists of two NVHB and one NVHP member. A Steering Committee, composed of delegates from each of the seven haemophilia treatment centres, the NVHP and the Dutch Hemophilia Nurses Society (NVHV), is responsible for monitoring that the registry data is appropriately used, as described in a governance document from the Medical Ethical Committee of the Leiden University Medical Centre.

MRDM, a company specialized in medical data management, was contracted to build, host and support the registry and app. This company hosts several registries, such as that of the Dutch Institute of Clinical Auditing, which monitor outcomes of, for instance, surgical interventions. The registry for haemophilia is their first registry that requires a lifelong follow-up.

The goal of the HemoNED Foundation is to use the registry to obtain data on number of patients, diagnoses, treatment intensity and outcomes, as well as for benchmarking & central registration of side effects of medication. Adverse events reported in the registry will be uploaded to the European Haemophilia Safety Surveillance System (EUHASS) database and also shared with the Netherlands Pharmacovigilance Centre, Lareb. HemoNED and the digital infusion log will generate data on the prevalence of haemophilia and associated disorders in The Netherlands, the efficacy and safety of treatment, the incidence of bleeds, and the occurrence of side effects from treatment. Obtaining such information will lead to improved quality of patient care.

Where are we now?

We are at an exciting point as the database has finally been built and we are now ready to include patients, once they have given informed consent. The Medical Ethical Committee of Leiden has no objections to the registry, meaning that it is not considered to be research and therefore, is not subject

to legislation for research with humans. Although in this setting informed consent is not required for a database of anonymised data, we still decided to inform all potential participants about the registry and ask them for their written consent.

Development of the digital infusion log

Patients will report infusions and bleeds via an app that has a web and mobile version. This data will be directly transferred to the registry via a secured internet connection. An overview of the app data is available for both patients and health care staff through a secured webpage and can be used in clinical practice during consultation. To make the app user-friendly, we formed two focus groups - one with doctors and nurses, the other one with patients, which were recruited via the electronic newsletter for NVHP members. Over 16 people responded to our request to take part in the development of the app. We enrolled a diverse group composed of parents of young children, young adults, adults and ageing patients. In these focus groups, we talked about preferences in registering infusions and bleeds and what kind of reports people would like to use. The analysis phase of the app is now 80% complete. During its development, a group of four so-called senior users will periodically be testing it out and reporting back any problems. Once developed, an acceptability test will be done by the full group of 16 people. We find it very important that the app meets the needs of patients, since it will be an important stimulant for discussion during doctor-patient consultations. The app will be offered to all people that use home treatment with clotting factors.

From an international perspective, it is important to work on harmonisation of registries within Europe and worldwide. The World Federation of Hemophilia (WFH) has recently published the dataset for the World Bleeding Disorders Registry. Another challenge is to start harmonisation of the various digital logbooks.

The landscape for novel therapies and monitoring of outcomes is changing rapidly. To have good data on the number of patients that would be in need of therapy is one of the requirements for their future success. At least that is one of the requirements for the so-called horizon scanning process that the Dutch Ministry of Health has started for first-in-class expensive medicines. With the HemoNED database, we have laid the necessary foundation to have this reliable data.

If you are interested to follow us or would like more information, please have a look at www.hemoned.nl. The website is in English as well.

Ascending Mount Hoverla in spite of haemophilia:

The personal experience of Igor Kovalov, MD, leader of the Poltava Regional Chapter of Всеукраїнське товариство гемофілії, the EHC Ukrainian NMO. Igor has severe haemophilia B.

By Igor Kovalov, translated by Sergiy Shemet, members of EHCs' Ukrainian NMO

"With this article, I want to share my experience of ascending Mount Hoverla, part of the Carpathians Mountains. At 2,061 metres above sea level, it is the highest point in Ukraine. My hope is that this story will encourage and be useful to those readers considering taking on a similar adventure."

At the time of embarking on this journey (July 2015), I was 54 years old and had numerous severe arthropathies of major joints, including both knees, making only slight to-and-from movements possible. This was the result of previous hemarthrosis and lack of adequate treatment in my early years, as only fresh frozen plasma was available in the USSR for the treatment of hemophilia B. It was this situation that shaped my interest in medical science, leading me to become a medical doctor and laboratory scientist.

In theory, my climb of Mount Hoverla began 10 years earlier, in 2005, when our close-knit family (my wife, two of our sons, our daughter-in-law and I) started to explore the beautiful Crimean Mountains. As they consist of three long, but low, ranges and my joints were in a much better condition then, this travel was not too difficult for me. Rather, it was at that time that I realised that I find great pleasure in such outdoor activities, as they helped me to overcome frustration and feelings of limitation imposed by haemophilia.

Those expeditions made me want to further challenge myself and make an attempt to conquer Mount Hoverla. I had many discussions with professional tourists who were familiar with the area, allowing me to assess the possible difficulties that would arise for a person with haemophilia. We determined that the best month for the climb would be July, giving us the most days with good weather. It was obvious that we wouldn't be able to join a large group of climbers, as I have my own rhythm of movement and needed plenty of pauses for rest.

And so, with a plan in mind, we first settled in the town of Yaremche, one of most picturesque places in the Carpathians. That morning, I infused myself with 1,000 IUs of factor concentrate and took a non-steroid anti-inflammation pill to alleviate joint pain. At 7 o'clock, we drove by car to the base camp *Zarosliak*, the most convenient point to start ascending. For safety reasons, we had to provide our

personal data at the checkpoint in the Carpathians National Park. At the base camp, we rented ski-sticks, which were very helpful on the narrow pathways. Sport shoes, sweaters and a rain coat are a must-have, as the weather in the Carpathians changes rapidly throughout the whole day. Tourists need to have one liter of water per person, and sandwiches, but it is recommended to have a light snack only once reaching the top of the mountain.

There are two routes from the *Zarosliak* base camp toward the Hoverla range. We decided to take the shorter but steeper one (Blue-White Route, 3.7 km) and to descend through longer but more shallow grounds (Green-White Route, 4.3 km) in order to have the opportunity to see the Carpathians from different sides. It's difficult to stray in the mountains, as both routes are well marked and there are usually lots of peoples on the pathways.

Our chosen route began in the forest. In many places, we had to walk over thick tree roots and the ski sticks proved to be exceptionally helpful.

After some time, the pathway led us to the plateau, where we took some time to rest. This is the last point with signs of civilization. Here, we met a group of children and their instructor, who advised us to drink water in small sips, just to moisture the mouth, as otherwise the water will weaken the body rather than stimulate it.

In many places, there were spring-wells with clear water, which had carved their way through the mountain.

We continued on the pathway, which was outlined with bushes of juniper plants. We eventually arrived at plateau (*polonyna*) Zarosliak, where you can admire the surrounding mountain peaks, though Mount Hoverla was still not visible from there. At this level, the plants are very diverse and flowers are amazingly colorful.

Here the ascending of 'Hoverla shoulders' begins. At every turn, you think you see its main peak, but then realise there are many others hide behind it…

A striking beauty of surrounding mountain panorama evolved with each new peak. Far-far below, we saw the red roofs of the *Zarosliak* camp – the beginning of our journey.

It began to rain several times, immediately making the pathway extremely slippery. This is one of the reasons why you need to choose a shallower route for descending. However, the rains didn't spoil the beauty of the landscape or our good mood. I remember one moment when we walked through the clouds. It was absolutely breath-taking!

At the steepest parts of the route, I had to be assisted by my companions, but for the most part, I used the tactic developed earlier in the Crimean Mountains: I first grab on to the big stones for support, while my legs go around the edge of the pathway.

Later, while descending, I needed to go hind ward with my legs in front of my hands.

The last portion of the way is the most difficult. We stopped every 7-10 meters to catch our breath due to the low concentration of oxygen.

Compared to lower horizons, the vegetation is very scarce here. There are no flowers, only low grass and stones.

And finally, here it is – the summit of the Ukraine!!! We were overwhelmed with incomparable feelings of victory, as we overcame our own fears. It was a sense of liberty and honuor, mixed with sentiments of joy that you get from leading a full life.

We were fascinated and screaming with delight.

Surprisingly, it was full of people at the top. It was an atmosphere of euphoria: everyone was hugging one another and singing songs and the National Anthem. Just celebrating life…

There are stunning sceneries everywhere. From this point, you can see the territories of Slovakia and Romania.

The descent was more difficult for me and took a longer time. However, we were full of adrenaline and still no challenge was able to spoil our great mood.

There were fewer flowers, but more well-springs on the Green-White Route. This is the place where the Prut river originates, before flowing further into the Ivano-Frankivsk and Chernivtsi regions, Moldova, Romania and falling into the Dniester river in the Odesa region.

At the bottom, we entered thick forests you only read about in fairytales.

All in all, the ascending took 4 hours and the descending took 5 hours and a half, 2.4 times longer than average time. We returned to the Yaremche after midnight.

For the next three days, I felt an unusual surge of energy, and there was no pain in my joints!

Upon returning home, my uncle jokingly asked me if I am going to climb Mount Everest next. Who knows, I said, 2 km and 8 km are rather comparable figures. If you have the desire, there are only a few things you can't achieve!

** More details of the trip along with contacts of Igor could be found here:*
http://www.hemophilia.com.ua/news/78/2371/

*** To give a similar opportunity to all people with haemophilia, the Ukrainian Haemophilia Association organises a summer camp in the Carpathian Mountains, where children with haemophilia will camp in the forest and ascend Khomiak Mountain (1,542 m). We warmly invite children with their families from all countries to join us! (See more on following page)*

Summer camp for children with haemophilia and their families in Ukrainian Carpathians

The Ukrainian Hemophilia Association (UHA) invites children with haemophilia and their families to participate in our Camp of Winners, being held from 15–20 June, 2017, in the picturesque and wildlife-rich Ukrainian Carpathian Mountains near the town of Iaremche. Teenagers (10–16 years old) will live in a campground, prepare their food at campfire and engage in various arts, creative and physical activities, including ascending mountain Khomiak (1,542 m) under the supervision of a coach, psychologist and a nurse. Parents can choose from different variants of accommodation in neighboring villages and be nearby, but do not participate in the camp.

We plan to make this an annual event. Its purpose is to demonstrate that children with haemophilia who have access to factor concentrate can live a normal and successful life, overcome any challenges and become winners. Participants from all countries are invited!

Contact: Victor Kronykh, Member of the Board, Leader of Youth Wing of UHA, fifan90@bk.ru

Link to photos of the 2016 Camp:
http://www.hemophilia.com.ua/news/79/2131/

1st APH Meeting of People with Haemophilia and Inhibitors

By Miguel Crato, President of Associação Portuguesa de Hemofilia e de outras Coagulopatias Congénitas, EHC Portuguese NMO

Lisbon, January 21, 2017

The existence of inhibitors in haemophilia is one of the major problems in this pathology because it puts in question the current treatment with factor concentrates.

It has been recognised throughout the international community that people with haemophilia (PWH) and inhibitors have a higher risk of bleeding, fewer treatment options and more muscle-joint damage.

This situation has a serious impact on the quality of life of people with inhibitors, leading to a more frequent isolation from the rest of the community, as well as to higher rates of work and school absenteeism.

For those reasons, the Associação Portuguesa de Hemofilia (APH), EHC's Portuguese NMO, knew that it was time to hold an event specifically for PWH and inhibitors.

The meeting was organised in Lisbon last January and was attended not only by people with haemophilia and inhibitors, but also by their relatives, nurses and doctors. Designed to be both informative and interactive, the day was full of various types of activities.

After welcoming participants and introducing them to one another, the first workshop took place on the theme "The past and the present of haemophilia and inhibitors." Participants were divided into working groups in which they were able to share their experience with inhibitors and what difficulties they face in having the condition, both in terms of treatment and in social, psychological and family interactions. At the end, all groups came back together to discuss the identified problems.

In the afternoon, three doctors were invited to take part in a medical panel that addressed some of the most important aspects of haemophilia and inhibitors.

The topic "Surgical intervention in people with haemophilia and inhibitors" was presented by Dr Margarida Santos, orthopaedist at the Centro Hospitalar Lisboa Central-Hosp. Curry and Cabral. She focused on the existing therapeutic options with regards to surgical procedures and other interventions.

Dr Santos, Dr Diniz and Dr Catarino lead a discussion on important inhibitor topics

Dr Maria João Diniz from the Hospital Central Lisboa-Hospital São José, addressed "Eradication of inhibitors through immunotolerance," a theme that is relevant, especially to parents of children with inhibitors. This procedure is the only one known to be capable of eradicating inhibitors, but remains a challenge within itself as it is expensive, time-consuming and sometimes unsuccessful.

To complete the discussion, Dr Cristina Catarino from the Hospital Center Lisboa Norte-Hosp. Stª Maria spoke about "New therapeutic approaches in people with inhibitors." She stressed that, in fact the future looks promising as new molecules currently in clinical trials are offering more perspectives for better treatment.

The topic was a natural lead to the second workshop, which was dedicated to "The future of people with inhibitors." Again in working groups and drawing on the conclusions made during the first workshop, participants formed discussions around the realistic needs of PWH and inhibitors. They developed methods to address their condition, both individually and within their relationship with the community. Each working group was invited to deliver a plan on creating a support group for PWH and inhibitors within the NMO.

Miguel Crato, President of the Portuguese NMO, explains to participants the workshop they will take part in

Upon reflection, the meeting was a rewarding experience and an opportunity for APH and the participants to share their specific problems with others who are facing a similar situation. Most importantly, proposed solutions to certain challenges will be taken and turned into real community action.

Feature Articles

Researcher spotlight: Professor Roger Schutgens

Interview by Raia Mihaylova, EHC Communications Officer

Roger Schutgens

In this column, we explore the work of a researcher involved in the area of haemophilia and other rare bleeding disorders.

It is exciting to be able to introduce Professor Roger Schutgens in this issue, as there is little (if anything) he hasn't done – a whole paragraph is needed just to list his various roles! He is a haematologist and epidemiologist at the University Medical Centre (UMC) in Utrecht, Netherlands, as well as head of department at the Van Creveldkliniek – one of the largest treatment centres in the Netherland for children and adults with haemophilia and other rare bleeding disorders. Since December of last year, Roger is also a professor of thrombosis at UMC. He chairs the Blood Transfusion Committee and the Anticoagulation Committee and is a member of the European Working Group (ADVANCE) for older haemophiliacs.

With so many titles and interests, there is no average day for Roger Schutgens. In this interview, he shares what keeps him up at night, what surprises him about haemophilia and some advice to ageing haemophilia patients.

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

When I started my work in haemophilia, I focused on the ageing haemophilia patient because they have the added challenge of age-related comorbidities like cardiovascular disease. Ten years ago, this was a topic that was just emerging and there were hardly any publications on it. It remains my interest area today. We have set up some large trials to gather knowledge on how we can better treat our ageing haemophilia patients. That's one part, the second part of my focus is haemophilic arthropathy, or looking into the reasons why blood in the joint is so bad for the joint. We have a long-standing research group focusing on that topic. Last year, we set up our own mice model to study the targets, which are really important for this damage, in order to find new targets for intervention. And the third thing is that I have recently become involved with patients with platelet disorders. All of my research questions come from the needs of the patients that I see at my outpatient clinic, including families with congenital bleeding disorders that have no diagnosis, which is very frustrating. That is the reason why I set up a large national study for innovative diagnostic strategies for patients with platelet disorders. That's the part I like the most: trying to find answers to questions patients have. Sometimes, we easily get the answers and other times it's a long road.

What does your average day consist of?

There is no average day. There are four areas that I spend my time on, divided throughout the day. First, of course, is patient care, seeing patients, talking with them. We have a very close comprehensive care team and spend most of our time on patient care. The second thing I do is management. I am head of the Van Creveldkliniek, which is one of the largest haemophilia centres in Europe, and that demands a lot of effort in finance and personnel management. The third thing is Ph'Ds. I have 13 PhD

students, which means 13 different projects. And fourth is education. I strongly believe that education is very, very important and people should not stop looking for knowledge, even when you are an established doctor or caregiver. I want to keep raising awareness for rare bleeding disorders, so I spend a lot of time in educating students, doctors, trainees and general practitioners.

What keeps you awake at night?

Fortunately, I am a very good sleeper, so there is hardly anything that keeps me awake. But I do worry about things. The case that currently makes me worry the most is one that involves a patient of mine, who has haemophilia and inhibitors. He is a young man with four children, who has a long-standing inhibitor and a very low quality of life. He is in a lot of pain. I have been looking for a long time for strategies to get rid of his inhibitor. So far, none of our attempts have succeeded, so I am forced to take different approaches, even testing out new products. These are difficult decisions to make as a physician, because these are new products, new by-passing agents that have not been tested much. Yet, you really want to change the life of this single patient because you know he can do better. It's a very responsible job to make such decisions. So that is what worries me: am I doing the right thing? Can we do better? Those are the basic questions you have with all the patients. And, fortunately, most of the time I can confidently say 'yes, we are doing the right thing.' Sometimes, it is really complicated but that's why the interaction we have with our patients is so important, it's not a one-way decision. I strongly advocate joint decision-making, where the patient's wishes and desires are taken into account.

When starting out, is there something that surprised you about haemophilia and/or other bleeding disorders?

Yes, and there are still things that surprise me today. First, we already know that prophylaxis with clotting factor is better than on-demand therapy for haemophilia. Yet, there are still ongoing trials comparing prophylaxis with on-demand. We should stop doing that, as it is just trying to find out the answer to something we already know.

The second thing that surprises me is that we still do not know what is the best optimal prophylactic regiment for our patients – when to start, how to start, which dose – we still don't know. And there are so many unknown things about product type and inhibitor development, inhibitor clearance, even after so many years of research. Ten years ago, we had the same questions as we do now.

What is the obstacle, that there is not enough data?

Yes, I think that is the main problem. You cannot get an answer if you do not have solid, big homogeneous populations. That is the main problem we have in all rare bleeding disorders.

Taking you back to the beginning, how did you get involved in this field of work?

I was involved with clotting disorders from the very first start of my career. During my traineeship, I did a thesis on laboratory diagnostics on venous thrombosis. So, clotting issues were always my interest, since I was very young. In the very beginning, I spent years on researching thrombosis but the more I learned about the other side of the clotting disorders, the bleeding disorders, the more fascinating and interesting that topic became to me, from a molecular point of view, but also from a patient point of view, to deal with hereditary diseases. So about ten years ago, I made the switch from the thrombotic part to the bleeding part.

If you weren't working on this, what would you like to do?

I would never change profession. That's the nice thing, from the age of four, I knew I wanted to be a doctor, and that is what I became. During my training as a doctor, I knew I wanted to become a haematologist, and that's what I did. During my training as a haematologist, I knew I wanted to go into clotting, and that's what I did. So, I am actually doing exactly the things I always wanted to do. I cannot imagine doing anything else. Sometimes, I think I want to build cars. But that's a completely different story. If I weren't a doctor, I would do something with cars and bikes.

What is one piece of advice or recommendation you would give to the ageing haemophilia patient, in the sense of how to live their life to the fullest?

My advice is for patients to live their lives without concern. You can do that, you can live without concern, because you can gather knowledge, you can make sure you know what to do, know where to go, know what to take, know who to call, no matter where you go. I think you can be in charge of your life with haemophilia, of course you can, and try to be the boss, take it as it comes but be sure to manage it. I don't think you have to live in fear or live with a lot of concern. Be well prepared, know what to do, create a strong relationship with your health care provider, with your doctor, with your haemophilia clinic. Talk to the nurses, talk to the physio, and talk to the social worker and to your doctor on how to best live your life with this disease. We have so many examples that we can do exactly that!

By Raia Mihaylova, EHC Communications Officer

The Lighthouse Project is an exciting and important new area of work in Ireland. Feargal McGroarty is the National Haemophilia System Project Manager at the St James' Hospital in Dublin, Ireland. He has long been involved in using GS1 global standards for application of barcodes that scan and track medications, which is one of the main components of the Lighthouse Project. We spoke with him to find out more details about the Lighthouse Project and its aims.

Relationships are the movement of life – they can hold us back or push us forward. Good, bad, difficult or easy, they always have an impact and can affect our mental and physical health. For haemophilia, one such relationship is between patient and doctor. Experience shows that the stronger it is and the more involved the patients are in their own care, the better the outcomes. While this is stating the obvious, many people, both on the patient and doctor side, still undermine the critical role the relationship plays in treatment quality.

It is exactly this concept that is at the heart of the Lighthouse Project. Designated by the Health Service Executive (HSE), Irelands' department of health, and in collaboration with, amongst others, St James' Hospital, its focus is divided into three clinical disciplines – epilepsy, haemophilia and bipolar disorder. The Haemophilia Lighthouse specifically will involve working with the haemophilia community in Ireland to further develop an electronic health record (EHR), providing all treatment centres with access to a national registry and a way to manage their patient files electronically. It will also include a Patient Mobile Application through which, by using barcode scanning, patients can ensure their medication is safe to take, record their treatment usage and report any incidents, all of which will improve patient safety and outcomes. Patients will have more control over their own medical records and access to their test results, as well as the ability to directly book their doctors' appointment. They are also provided with the option to communicate and share their records with family, caregivers and any health care provider they see fit, such as their general practitioner.

The patient will see you now

The Haemophilia Lighthouse project will be led by Dr Barry White, who is the National Haemophilia Director on behalf of the National Haemophilia Service of Ireland. This service is based in the National Coagulation Centre (NCC) at St. James's Hospital. Dr White is previously responsible for implementing

a national haemophilia electronic patient record and has instigated a change in service delivery by improving the way that medication is delivered to the patient from the NCC and other centers around Ireland. This service layered international global standards – GS1 standards – that allow for barcoding to load the track and trace of the medication as it goes to the patient.

He then realised that the next logical step to the service is to empower the patient with control of their own medical records and appointments. This was the beginning of the idea for the Lighthouse Project. It builds on to Dr White's view that we need to get to the point where it would be that "the patient will see you now," rather than the other way around.

Vision for the future

The overall aim of the Lighthouse Project is to achieve improvements across the key domains of healthcare delivery: quality and safety, experience and cost effectiveness. It will serve as an example of the benefits such a platform brings to healthcare, which is also how it got its name. As Feargal McGroarty explains:

"Lighthouses are structures that are dotted along the coastline and act as a beacon for ships so that they can be guided safely to their next port and avoid hitting rocks or perishing on sand banks. This project is to be a beacon for other areas within the health service, to learn from, to stay safe, so that best practice can be adopted by any other service that chooses to use it."

The project is in the process of going through formal tender procedures for suppliers and is awaiting final funding approval.

My experience with desmopressin

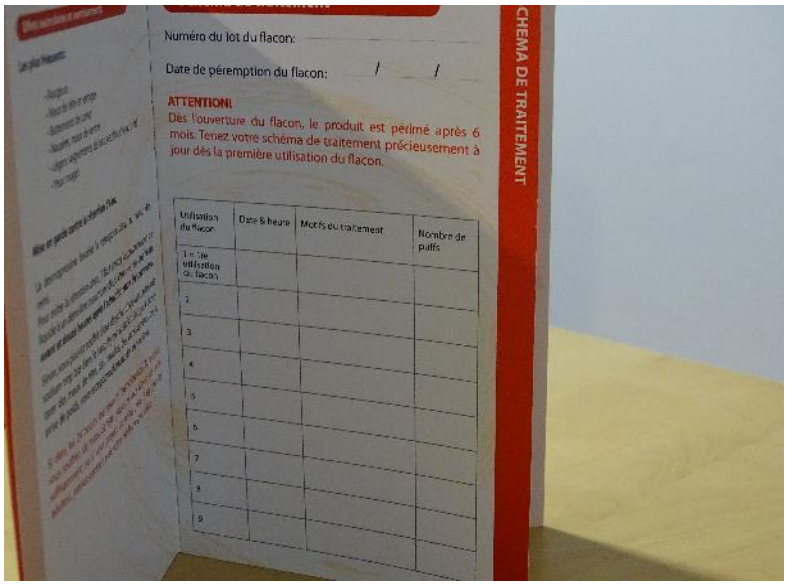
By Jo Eerens, EHC Membership Officer

2017 marks 40 years since the discovery of desmopressin. Prescribed as treatment in mild to moderate haemophilia A and von Willebrand cases, desmopressin stimulates the release of von Willebrand factor and increases its levels by three to five – fold. A quick Google search shows its various uses but we bring you a first-hand account of how this drug affects the life of a mild haemophilia patient.

Desmopressin had already been in use in many countries, such as Sweden, for twenty years before it became available in the form of a nose spray in Belgium. It was thanks to the continuous efforts of our haematologists, the Ministry of Health and the Reimbursement Organisation that it was finally allowed on the market here in 2010.

As a mild haemophilia patient, I was quite surprised that for the first time in my life – I was 55 years old at the time – I had a drug that protected me from accidental bleeds. I am at a risk of nose bleeds for periods of time only once or twice a year and I have never had a spontaneous bleed in my joints. It has always been by accident or in surgery.

No matter where I go in Belgium, the nearest treatment centre is always within about 50 km. Therefore, I don't need to carry my desmopressin medication every time I leave home, but I always bring it when I travel abroad. This way, I'm certain to have a drug that helps enormously in stopping my accidental bleeds or those caused through injury.



The desmopressin nasal spray comes with an informative booklet where patients can log each usage

I also use it when I visit my dentist, since the risk of a bleed is high when getting teeth work done. This way, I'm prepared and don't have to ask for replacement therapy. For one, this is very cost effective.

Although there are some very small inconveniences, such as possible headaches or not being able to drink water, these are minor problems in comparison to otherwise bleeding for eight days after a dentist visit.

In Belgium, all mild and moderate haemophilia A and von Willebrand patients were asked to visit their haematologist in order to record if they experienced any side effects with desmopressin. Nothing serious was reported and any patient can use the drug to prevent or treat a bleed when it occurs. I really must admit – this gives me a great feeling of safety and I am very grateful that it exists.

No stone unturned at the 10th EAHAD Annual Congress

By Dr Radek Kaczmarek, EHC Steering Committee member

The 10th Annual Congress of the European Association for Haemophilia and Allied Disorders (EAHAD) was held in Paris from 1-3 February. EAHAD is a multi-disciplinary association of health care professionals interested in haemophilia and other bleeding disorders, and has collaborated with the European Haemophilia Consortium (EHC) on various projects. The two organisations have a memorandum of understanding with each other and naturally cross-fertilise on ideas, insights and efforts.

The conference sessions covered an impressively wide range of topics, going far beyond coagulation focused themes in an attempt to answer elusive or inevident questions on statistical analysis of clinical data, pain management, immunogenicity, extended half-life products, gene therapy and other non-factor replacement therapies.

In the opening session, Amanda Bok, CEO of the EHC, discussed the work and impact of the European Commission Expert Group on Rare Diseases on the haemophilia community. As one of the patient organisations belonging to the group, she explained how the expert group works together with various stakeholders, such as regulators, Member State representatives, European Commission officials and others, in order to develop policies and to support people with rare diseases, amongst others, by laying the foundation for European Reference Networks (ERNs). Following Amanda's talk, Professor Mike Makris, Director of the Sheffield Haemophilia and Thrombosis Centre, Sheffield, UK, discussed the ERNs in more detail, now that they are coming into fruition after many years of development. Spurred by the European Commission and the Expert Group, we now see the creation of 24 thematic networks, covering over 5,500 rare disorders, based on applications of interested parties. One of the ERNs, named EuroBloodNet, covers haematological diseases, including inherited bleeding disorders, and got its official approval in December 2016. Professor Makris envisages that EuroBloodNet will be an opportunity to build on the achievements of the EUHANET project.

The next session was devoted to immunology. Professor Marijke van den Berg presented important data showing that vaccinations shortly before or after factor VIII administrations do not raise the risk of inhibitor development, contrary to a common belief stemming from the so-called danger theory of inhibitor development. According to this theory, any challenge to the immune system, be it infection or vaccination, coinciding with the factor replacement therapy may lead to eliminating the administered factor by directing 'friendly fire' at the infectious agent/vaccine, etc. This belief has complicated the management of haemophilia in paediatric patients, who, as all their non-haemophilic peers, should be vaccinated to prevent life-threatening or debilitating infections.

The key points of the pain management session were that treaters in general tend to underestimate patients' pain and that guidelines on pain management in patients with bleeding disorders are lacking. Yet, approximately one third of patients with haemophilia report chronic pain, making it a serious health problem.

Many interesting developments on coagulation factor X were shown. In the coagulation cascade, factor X is located downstream from factors VIII and IX, whose joint activity is necessary to activate factor X. Therefore, one would anticipate that administration of already activated factor X, instead of the missing factor VIII or IX (or to by-pass an inhibitor thereof), could be a way to treat haemophilia. Yet, this method has been ineffective because activated factor X is quickly inactivated by its own natural plasma inhibitors when away from the bleeding site. Dr Olivier Christophe, who has worked on ways

to overcome this limitation, presented data on variants of recombinant activated factor X that are less susceptible to inactivation and could be used in treatment of haemophilia, including inhibitor patients.

A highly-anticipated session was on future perspectives in treatment. It featured talks on most of the new therapies and an excellent summary of the progress in gene therapies for haemophilia. One of the many important issues raised in relation to non-replacement therapies was the risk of effects from combining these drugs with standard products. Since some patients in clinical trials of non-replacement therapies experienced bleeding episodes that required treatment with standard products, the risk of synergistic action of these formulations should not be ignored.

The talk on gene therapies included some impressive results, like achievement of stable factor VIII and IX levels of >140% and 20-44%, respectively, but also raised many important questions. Can factor VIII or IX expression be life-long in patients after gene therapy? Can gene therapy be used to prevent inhibitor development? Will the viral vectors used to introduce the correct factor VIII encoding gene prove ultimately safe in the long term?

It will take a while before these questions can be answered, but in the meantime, there will surely be a lot to be excited about.

Second Multi-Stakeholder Symposium on Improving Patients' Access to Rare Disease Therapies

By Laura Savini, EHC Public Policy Officer and Olivia Romero-Lux, EHC Steering Committee member

As every year, the European Organisation for Rare Disease (EURORDIS) held in February a policy event to mark Rare Disease Day, an international observance held either on the 28th or 29th of February to raise awareness on the issues faced by people affected by rare diseases. EURORDIS is an international umbrella organisation bringing together over 600 patient organisations working in the area of rare diseases, including the European Haemophilia Consortium (EHC). Through these events, EURORDIS gathers stakeholders, such as its members (i.e. patient representatives), regulators, policy makers, representatives from national government agencies and the pharmaceutical industry to advance discussion on the rare disease policies.

This year's event mainly focused on how to ensure that novel therapies are developed and that they reach patients in a speedier manner.

EURORDIS has long been working with both public agencies and the private sector towards this objective. For instance, in 2016 EURORDIS was instrumental in the creation of a rare disease committee within the United Nations and in the same year, Mr Yann Le Cam, EURORDIS Chief Executive Officer (CEO) was nominated to sit on the board of the European Medicines Agency (EMA) as a patient representative. The list of activities led by EURORDIS is long and touches upon many domains, including patient training, ensuring patients' views are properly taken into account into European and international rare disease policies and projects, and developing tools to connect patients affected by extremely rare conditions. The list goes on. However, one of the main challenges faced by people with rare diseases, and one of the focal points of EURORDIS' work, is access to treatment. Difficulties in accessing orphan medicinal products are caused by multiple factors feeding into each other and creating a vicious and endless circle. For instance, one of the main barriers to developing treatment is the small size of the patient population that can be enrolled in clinical trials, which in turn hinders generation of high quality data for efficacy and safety. This situation often discourages regulatory and reimbursement agencies to license and reimburse the product, which in turn limits investment because of the uncertainty of returns.

EHC CEO Amanda Bok spoke on the European Reference Networks (ERNs) at the symposium

During the symposium, participants were reminded that an extremely high number of patients with rare diseases die before reaching the age of five and therefore, it is high time to find a solution to both ensure that patients can access existing therapies, and to encourage and facilitate the development of novel orphan medicinal products. In the last couple of years, the EMA has been developing new regulatory schemes, such as adaptive pathways and Priority Medicines (PRIME), to ensure faster access to novel medicines targeting unmet medical needs. However, the problem in access to novel medicines does not only lie with regulators and needs addressing at all stages of the medicines lifecycle development – from research and development (R&D) to market access and post-marketing surveillance. It was therefore with great excitement that EURORDIS unveiled a new

reflection paper: "Breaking the Access Deadlock to Leave No One Behind – a contribution on possibilities for patients' full and equitable access to rare disease" to rethink the whole orphan medicinal product development model.

The paper establishes that some regulatory tools, such as the Orphan Medicinal Product Regulation (OMPR), have provided incentives for investment, but it should be noted that even if a product gets licensed, it will face roadblocks for national pricing and reimbursement. This is because payers are unwilling to invest in products that have greater uncertainty and manufacturers need to recuperate high R&D costs and know that their product will be only used in a very small number of patients. EURORDIS proposes a new model in which there is greater collaboration between developers of novel medicinal products, regulators and payers. This is to ensure that data satisfying both regulatory and reimbursement requirements can be generated early on. The paper also encourages greater European cooperation to determine value and re-visit traditional pricing models and to ensure that price is adapted to the purchasing power of each country. Finally, European Reference Networks (ERNs) should be harnessed to generate continued evidence on treatment outcome in rare diseases.

Laura Savini, EHC Public Policy Officer, Amanda Bok, EHC CEO, and Olivia Romero-Lux, member of the EHC Steering Committee at the EURORDIS Symposium

During the event, Liven Annemans, who led the European Working Group for Value Assessment and Funding Processes in Rare Diseases, presented nine new recommendations that were elaborated to help improve the consistency of pricing and reimbursement for orphan medicinal products in Europe.

In the closing remarks for the Symposium, Yann Le Cam announced the imminent formation of a new multi-stakeholder group that will draft a One Text Collaborative Plan of Action for all parties to collaborate in a process to improve patients' access to medicines. This process will aim to enhance and sustain trust between the various stakeholder groups, a fundamental condition to achieve better patient access to medicines.

The proposed way forward is ambitious and seeks to improve access to treatment, something that is very close to the EHC's heart. We were energised by the two days of novel ideas and hope to contribute towards the success of this new project in any way possible.

The next EURORDIS event, where the European Haemophilia Consortium (EHC) will be represented will be the EURORDIS Membership Meeting coming up later in May in Budapest.

Announcements

EHC events

June 27	EHC Round Table on Orthopaedics Aspects in Haemophilia Open to NMOs only and selected participants
June 29-July 2	3rd EHC Leadership Conference – Open to NMOs only *Brussels, Belgium*
Sept 14-17	EHC Workshop on Tenders and Procurement – Open to NMOs only *Sofia, Bulgaria*
Oct 6-7	EHC Annual Conference – Open to all *Vilnius, Lithuania*
Nov 16-19	EHC Workshop on New Technologies in Haemophilia Care – Open to NMOs only *Lisbon, Portugal*
Nov 28	EHC Round Table on Extended Half-Life Factor Concentrate Usage and Measurement Open to NMOs only and selected participants *Brussels, Belgium*
Nov 30- Dec 3	Inhibitor Summit – Open to NMOs only *Barretstown, Ireland*

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

Other events

July 8-13	International Society on Thrombosis and Haemostasis (ISTH) Congress 2017 *Berlin, Germany* – More information at http://www.isth2017.org/