EHC NEWSLETTER

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EHC Newsletter September 2017

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President and CEO Report

Welcome back from the summer holidays. We return to the office with energy and excitement about what has been accomplished so far this year and everything yet to come. If the length of this newsletter is any indication, there is much happening at the European Haemophilia Consortium (EHC), our National Member Organisations (NMOs) and our community. The developments in our field are not slowing down and neither are we.

Brian O'Mahony, EHC President

Amanda Bok, EHC CEO

In June, we held our second **Round Table** of the year on **'Orthopaedic Aspects in Haemophilia Care,'** during which multidisciplinary experts discussed haemophilia as primarily a musculoskeletal, rather than a bleeding, disorder, and presented multiple means towards achieving and maintaining joint health for people with haemophilia without and with inhibitors.

Shortly after the Round Table, a broader conversation took place as our community came together for the third **EHC Leadership Conference**, held in Brussels, Belgium. The success of this Conference grows each year as it brings the diversity of our NMOs into one place, enabling them to exchange information, best practices and experiences. Highlights from the four-day programme included a community exchange on gene therapy, women and bleeding disorders, extended half-life products, fundraising, engagement with industry, volunteer-staff collaborations, patient-clinician engagement, inter-generational dialogue, succession planning and using the Kreuth IV recommendations in national advocacy, amongst other things. The Leadership Conference also featured poster sessions and NMOs had the opportunity to exchange their best practices directly with their peers. We never cease to be amazed at how much we can learn from each other and already look forward to the next one.

The **EHC** was also an active participant of the **ISTH (International Society for Thrombosis and Haemostasis) Congress** held in Berlin, Germany**.** This year, we were delighted to attend not just as delegates but also with an EHC exhibition booth that highlighted our major areas of work and enabled us to share it with a large audience of haematologists, researchers and a variety of other medical experts (approximately 10,000 attendees). We also attended ground-breaking sessions that presented the latest updates on novel haemophilia treatments – from successful clinical trial results to gene therapy, you could feel the excitement of the scientific community about the future of care.

Looking ahead to the autumn and following on last year's **workshop** on '**Tenders and Procurement'** in Baku, Azerbaijan, we will hold the next one in this series in Sofia, Bulgaria, from September 15-17th. Experience shows that best overall outcomes are achieved when patient representatives and expert clinicians are involved in their national tenders for treatment. We believe deeply in this concept and have created these workshops to provide

all of our patient representatives with the necessary technical and practical know-how to actively participate in their national tender and procurement processes.

Another process we believe in deeply is the **Wildbad Kreuth** consensus process. Readers may remember that this process last met in 2016 and resulted in 12 recommendations, which we outlined in our April World Haemophilia Day event (see April newsletter). Since then, we have also been working on an animated advocacy video to highlight those recommendations and help disseminate them at all levels. Therefore, we invite you to stay tuned to the EHC channels; we aim to launch the film before the end of this year!

Speaking of films, we've also had the opportunity to continue our close collaboration with film director/producer Goran Kapetanovic, whom readers might remember from 'Haemophilia Stories' (see EHC website), and whose new EHC film, '**Inhibitor Stories'** will be launched at our EHC Conference next month, to be held from October 6-8 in Vilnius, Lithuania, and humanely captures the realities of life that our inhibitor community face day-to-day. In tandem with this, at our EHC Conference, we will also proudly **launch our new European Inhibitor Network (EIN) website** with dedicated sections for people with haemophilia and inhibitors, their caregivers and NMOs, as well as an online community platform. Of course our **EHC Conference** will feature much more than just the above, including a varied scientific programme (see www.ehcconference.org) and our energetic annual debate pitting youth against established leaders on a number of important issues. Finally, we are delighted to announce that this year we have formed a Medical and Scientific Advisory Group (**MASAG**) to ensure a truly multidisciplinary input into all of our work by bringing together experts from nursing, physiotherapy, paediatrics, lab specialists, gynaecology, psychology, epidemiology, regulatory affairs, health economics, genetics, hepatology, orthopaedic surgery, blood safety, immunology and the field of women and bleeding disorders. MASAG members will have their first broad face-to-face meeting in the margins of our Conference. The **EHC Conference** will be a varied and high-energy event, and we look forward to welcoming you all in Vilnius then!

After our Conference our work will continue with our annual **New Technologies workshop**, to be held from November 17-19 near Lisbon, Portugal. Gone are the days when haemophilia care only gets discussed in terms of factor treatment. With the birth of novel products, comes a paradigm shift and the relevance of the New Technologies workshop grows more significant than ever. Towards the last week of November and first weekend of December will see our last **Round Table** of the year take place, namely on '**usage and measurement of EHL factor concentrates and novel non-replacement therapies**,' immediately followed by our **Launch Event of the PARTNERS programme** to be held in, and hosted by, the European Parliament – with both events taking place on November 28th. We have been working hard on moving PARTNERS forward and have, since our last newsletter in April, held country visits in Ukraine, Serbia and the Kyrgyz Republic – and have further visits to Albania, Bulgaria, Romania, Latvia and Macedonia scheduled this autumn. We were also pleased to see PARTNERS receive the official endorsement of EURORDIS – Rare Diseases Europe and the European Patients' Forum (EPF), and to be close to finalizing our Memoranda of Understanding with corporate partners. Rounding off November, we will spend the first weekend of December meeting back in

Barretstown, Ireland, for our second annual **Inhibitor Summit** where we look forward to further building on last year's work and continuing to grow and strengthen our European community of people with inhibitors and their families and caregivers.

Finally, beyond 2017 lays a blank slate of possibilities as our current 2014-2017 Strategic Plan comes to a close and we launch a new one! To ensure a bold and meaningful new Strategic Plan that reflects the needs and aspirations of our European community and ensures the EHC continues to move in the right direction, we've hired an external consultant who will lead a targeted stakeholder consultation process this autumn and feed back into the EHC at the end of the year.

More details about all of the above are waiting for you in the next pages. We wish you pleasant reading and look forward to seeing many of you soon at an upcoming EHC activity!

EHC NEWS

Comprehensive care in haemophilia: Elective Surgery for People with Haemophilia and Inhibitors

Miguel Crato developed an inhibitor to standard factor treatment when he was twelve years old. His history with joint bleeds into the right knee began around the same time, causing reduced mobility and pain to be part of his daily life. With the goal of improving his quality of life and after years of weighing the risks versus the possible outcomes of surgery, Miguel decided to go through a knee replacement procedure in March 2017. His surgery was the first of its kind to be performed on a person with inhibitors in Portugal. Although joint replacements are routinely carried out on patients with haemophilia, inhibitors present a serious surgical challenge, as there is no standard protocol, bleeds are more difficult to control and optimal outcomes depend on many factors.

Miguel kindly shares his experience with the process, which starts with months of preparation and goes well beyond being discharged from the hospital.

Standing up straight – having inhibitors and going through a knee replacement surgery

By Miguel Crato, President of Associação Portuguesa de Hemofilia e de outras Coagulopatias Congénitas (APH), EHC Portuguese NMO; followed by an interview with Dr Margarita Santos, orthopaedic surgeon

Haemophilia with inhibitors is a condition that can be very hard to manage, especially when it comes to undergoing major surgeries. It is difficult not only for the person with inhibitors, but also for the doctors, as factor usage is ineffective and the only option to control haemostasis is using bypassing agents with a very limited time effect.

Decision

In my 47 years of life, I have had a long history of inhibitors and joint bleeds. Severe arthropathy had developed in my right knee already since my youth, slowly decreasing my quality of life because of pain, reduced mobility, inflammation, job absence, other joints being affected in a domino effect, etc. The simple action of starting to walk in the morning was usually a nightmare.

Given my history of health, the decision to go forward with a knee replacement surgery was not an easy one. It involved years of discussions with my haematologist, as well as with my orthopaedist, who would be the one carrying out the surgery. We exhaustively debated the pros and cons and when the final joint decision was made, there was a clear objective: improve my quality of life with a very well-planned surgery and haemostasis control.

Preparation

The involvement of a multidisciplinary team was paramount, as was deciding which bypassing agent would be capable of offering me the best haemostatic control during and after the surgery.

To make the right choice, two types of blood tests were made after infusion of rFVII and aPCC (Activated Prothrombin Complex Concentrate) – a thrombin generation test and ROTEM® analysis: thromboelastometry. The results showed that for me, better results were reached with rFVII, with faster and more consistent clot formation. The decision was that for the surgery, and the following 15 days, I would receive rFVII; after that, I would be switched to aPCC.

It was also very important to evaluate my treatment history. Previously, I had had other major surgeries – cholecystectomy, a dental extraction and a synovectomy in one of my elbows. All of these had been treated with rFVII, despite the fact that my joint bleeds had been generally treated with aPCC.

In additional preparation, I started an intensive swimming physiotherapy programme one year before the surgery was to take place; I took extra precaution and care with regard to dental issues and I started to mentally prepare myself and my family for the very long road of recovery.

Of course, I clearly understood the risks of this kind of surgery, especially because this was the first knee replacement surgery performed on a person with inhibitors in Portugal.

Surgery

On March 20th, 2017, the surgery was successfully performed. No blood transfusions were needed and very good levels of haemostasis had been maintained. During the first 32 hours post-surgery, I was administered seven mg of rFVII every two hours, with the frequency of infusions gradually decreasing in the next days. Starting on day 10 until day 15, I needed only one transfusion so that I could participate in my physiotherapy sessions. In addition, I infused with tranexamic acid up until day 10.

I started physiotherapy 48 hours after the surgery and I was able to stand up on day three. Extra precautions were taken and the exercises were adapted to the fact that I have inhibitors.

Recovery

I was discharged from the hospital on day 15. Right after, I started doing physiotherapy three times a week, always with a prior infusion of aPCC. The objectives of the physiotherapy were to recover joint amplitudes, to promote kneecap mobility, to restore functionality of the soft tissues, as well as to gain flexibility and strength. Also, it was important to achieve motor control and adaptation to the new joint and body posture.

Since I have inhibitors, taking extra care during the physiotherapy sessions was crucial. It was necessary to have very open and frank conversations with my physiotherapist and to discuss shared objectives in order to reduce the stress from a slow recovery and to avoid bleeding and inflammation. To achieve these aspects, we held longer sessions, frequently used ice and took extra breaks, as well as going over good methods for home therapy and autonomy.

Results

Despite the slow recovery due to arthropathy in both of my ankles, the results are magnificent. Just after three months, the range of motion of my knee reached 0° in extension and 90° in flexion. Previous to the surgery, the extension was 25° and the flex 75°.

The pain in my knee has completely disappeared and for the first time since I can remember, I am able to stand up straight and have the feeling of being taller. Even while sleeping, I notice a huge improvement – I do not need to sleep on my side anymore and can sleep on my back without needing a pillow under my knee. Now, four months after the surgery, I can see a major improvement in my quality of life in all aspects.

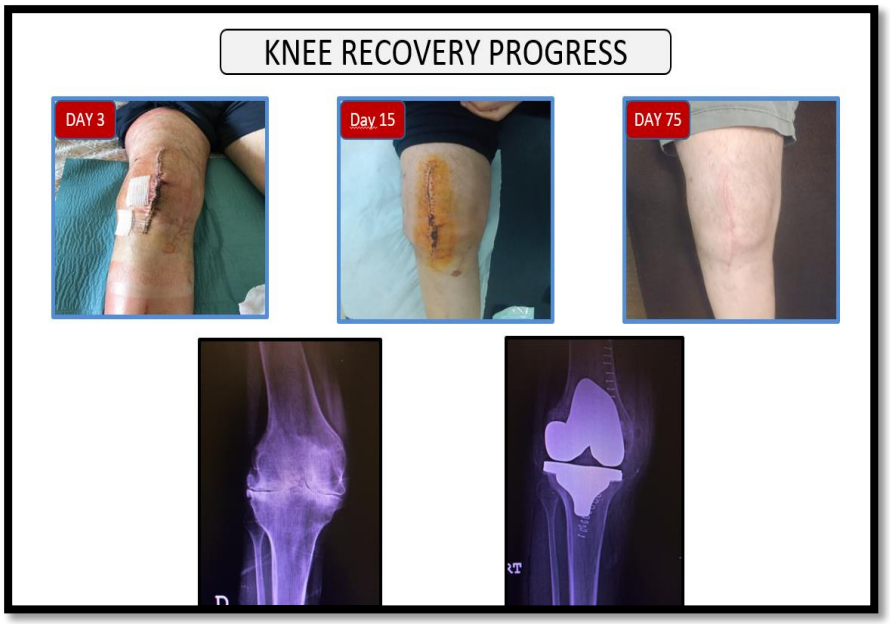

Photo courtesy of Miguel Crato

Conclusion

I must say that if you make a clear and informed decision together with the medical team involved in the surgery, if you choose the best bypassing agent for you, if you are prepared mentally and physically, and if you comply with the physiotherapy programme, there is no need to continue to watch your quality of life decreasing over the years.

On a global note, I understand that not all countries in Europe can offer the best conditions to perform this kind of procedure. However, it is essential that those who lead haemophilia treatment in Europe understand that people with inhibitors should not be condemned to a low quality of life. If the surgery is carried out by taking all the necessary preparations and under cover of bypassing agents, this can insure that a person with inhibitors becomes more cost-effective on the health budget of the country in the long term.

Miguel's surgery was performed by his orthopaedist, Dr Margarita Santos, of the Centro Hospitalar de Lisboa Central (C.H.L.C.) in Portugal. She works in the field of haemophilia and has several publications on the topic, including a chapter on haemophilia and orthopaedic problems in the book "Hematologia e outras especialidades." Since 1996, Dr Santos has been responsible for the orthopaedic treatment of patients with congenital coagulopathies.

As Miguel stressed in his account, it is essential that the decision of surgery is made after extensive discussions between the person with inhibitors and the involved multidisciplinary team. While the patient has to be sure they are motivated and mentally prepared for the hard recovery work that is to follow, there are many medical factors that the healthcare professionals need to consider before concluding if someone is a good candidate for joint

replacement surgery or not. Dr Santos shares that for her as a doctor, this process begins with the understanding that this is not just about the procedure, it is about a suffering person who has resorted to this step in hopes of a better quality of life.

Read more about the medical perspective of Dr Santos in carrying out a knee replacement surgery in a patient with inhibitors.

How do you conclude that someone is a good candidate for orthopaedic replacement surgery?

Dr Santos has been an orthopaedic surgeon since 1985

MS: A big influence is the titer, or the levels of inhibitor in the blood, and how the inhibitor responds to the bypassing agents. I try to understand what the patient expects from the surgery and what I can offer him. I look at the age of the patient and his psychological profile, which is very important in the recovery process. Other factors include physical parameters such as muscle mass, degree of joint degeneration and the deformities that already exist.

This was the first knee replacement surgery on a patient with haemophilia and inhibitors to be done in Portugal. What were your concerns?

MS: My major concern was clearly if haematologic stability can be achieved. The prevention of any type of infection and being able to correct the deformities without causing other complications are also essential for the success of the surgery.

In addition to joint replacement, what surgery options do people with haemophilia and/or inhibitors have that could lead to an improved quality of life?

MS: There are many surgical options to which the patient can resort to, but the most frequent ones are synovectomies, tendon stretching, correction of deformities, arthrodesis and, of course, arthroplasties.

What kind of questions should the patient ask, what do they need to know?

MS: First, the patient should be educated on the risk of the surgical procedure and what exactly a knee prosthesis is. After being thoroughly informed and measuring the pros and cons, he should also consider his age, quality of life and if what we, as medical professionals, can provide is in line with his expectations. So, some of the questions he should ask are: *What risks do I run? Does my age influence the placement of a prosthesis? What is a knee replacement? What does the surgery consist of? Does my muscles mass allow me to have a good recovery?*

As the treatment of haemophilia (and inhibitors) requires a multidisciplinary approach, various specialists were involved in Miguel's knee replacement surgery. Dr Santos is joined by Dr Maria João Diniz from the Immunochemotherapy department of the Centro Hospitalar de Lisboa Central to provide insight into the usage of bypassing agents during such a procedure.

What is important to consider when choosing the bypassing agent to be used during surgery? Are there limitations to bypassing agents?

MD: As the response to bypassing agents vary among patients, we must consider the patient's previous clinical response to them and the availability of the products. The bypassing agents we have are less predictable, and thus more difficult to control and monitor.

Can you talk a little about the thrombin generation test (TGT) and ROTEM-Thromboelastometry? What are they and what do they determine?

MD: As the final product generated by bypassing agents is thrombin, a thrombin generation assay (TGA) can be used for the assessment of the properties of these agents, to monitor their in vivo thrombin generation capacity and therefore, to optimise the treatment. Thromboelastometry (ROTEM) is a test designed to assess clot formation.

The EHC expresses gratitude to Miguel Crato, Dr Margarita Santos and Dr Maria João Diniz for sharing both the patient and medical perspectives of going through a knee replacement surgery.

EHC Round Table on Orthopaedic Aspects in Haemophilia Care

By Laura Savini, EHC Public Policy Officer

The EHC Round Tables of Stakeholders strive to bring together different stakeholders to present the latest information and generate engaging discussions on current issues in haemophilia. The full report of the event is now online on the EHC website (www.ehc.eu) but here we give you a few take-away messages from the discussions.

Round Table on Orthopaedic Aspects in Haemophilia Care, June 2017

Prophylaxis, prophylaxis, prophylaxis….

The key to maintaining a healthy musculoskeletal system is to prevent bleeds and we now know that the best method to do so is prophylactic treatment. There is no set way of doing prophylaxis and the regimen will largely depend on each patients' family history, genetic profile, occupation and aspirations with regard to sport practice. One of the key problems in prophylaxis is adherence. Many patients find it burdensome to infuse numerous times a week, plus it can have some side-effects, such as vein damage. The take-home message from the clinicians present at the event is to define the prophylactic regimen together with the patient, and to ensure patients fully understand the concept of peaks and troughs and how they protect against bleeds. Another take-away message: do not stop prophylaxis in adults or you'll lose all the investment made in them as children.

Patients: Have your say!

Member of the European Parliament Dr Miroslav Mikolasik joined the Round Table to express his support to the rare bleeding disorders community

This is a phrase that was stressed again and again and though it seems like stating the obvious, it is often overlooked. Patients need to have an active role in their health care decisions. To do so, it is important that they understand their condition and how various treatments and therapies will impact them. The most compelling example was given by Mr Miguel Crato, EHC Steering Committee member, who explained in detail how he came to the decision to undergo orthopaedic surgery, even though he is affected by inhibitors (see pg. 06). He explained that when it comes to such a big decision, the patient needs to communicate with the whole medical team to make sure that he understands the process, but also to explain his preferences. Preparation is key. It is important that before such a major surgery, one is in the best physical condition possible and in a good mental state

through support from family and friends. In addition, the patient should play an active role in deciding which exact outcomes he wishes to achieve and these goals should be set together with the healthcare professionals over 'realistic' timelines. This will be beneficial for the recovery process.

No doctor is an island

Collaboration amongst various healthcare professionals, such as haematologists, physiotherapists, orthopaedic surgeons, nuclear medicine specialists and nurses, is vital. People with haemophilia and orthopaedic issues will have complex needs that can best be addressed by the expertise of different healthcare professionals working together.

The programme brought together a diverse group of healthcare professionals, such as physiotherapists, haematologists, inhibitor specialists and orthopaedic surgeons

Surgery: timing is everything

"Surgery needs to be done at the right time!" This was the message from Dr Luigi Solimeno, an orthopaedic surgeon working in Milan. The "right time" comes just when the joint is damaged enough to warrant a surgical intervention, but not enough that the surgery will no longer be beneficial. The idea is to do surgery when the benefits can be maximised.

Inhibitors: an unchartered territory, proceed at your own risk

Unfortunately, the health status of people with haemophilia and inhibitors is still poor compared to those with no inhibitors. This is because regular treatment no longer works and people affected by inhibitors are left vulnerable to bleeds. Furthermore, the handful of

treatments that currently are administered to people with inhibitors have a very short half-life. The option of inhibitor eradication or immune tolerance induction (ITI) is an expensive treatment that often cannot be afforded in lower-income countries. During the Round Table, the focus was mainly on surgery with inhibitors, which, as we found out, is a great unknown for healthcare professionals. In fact, it is very difficult to monitor coagulation factor levels during surgery, as the functioning of bypassing agents is still unclear and patients may react differently to different products. Besides the cost of the treatment, costs also arise from the increased monitoring of patients following surgery.

It's the small things that make a big difference

Although the event focused on the importance of prophylaxis in maintaining joint health, it was also underscored that many conservative treatments and techniques could bring considerable benefits to patients for a fraction of the price of replacement treatment. For example, regular practice of sports and physiotherapy will help with preventing joints bleeds by strengthening the muscles that support the joints. Ultrasound imaging is a cheaper alternative to MRI and can give a very accurate picture of what is happening in the joint and if the treatment regimen is working. This technique is particularly useful for sub-clinical bleeds, which may be difficult to identify through regular physical examinations. Another positive aspect of ultrasound imagining is that it can distinguish between actual bleeds and inflammation. This is important because coagulation factor treatment will not alleviate pain from inflammation and in this way, precious resources can be saved. This raised the point that there needs to be more discussions about the use of adequate anti-inflammatory treatment together with replacement therapy. Speakers also noted that treatment will neither restore a damaged joint nor ease the pain, so alternative procedures should be considered to alleviate pain and improve quality of life. One of these treatment options is radioactive synovectomy, which can reduce pain caused by an inflamed synovium.

Participants had the opportunity to ask the specialists questions and to share their own experiences

Additional resources

All presentations and the Round Table report are available on the EHC website. During the next EHC Annual Conference (October 6th-8th, Vilnius, Lithuania – see pg. 53), there will be a session dedicated to orthopaedic issues in haemophilia, as well as a discussion on surgery and physiotherapy in people with inhibitors.

The next EHC Round Table of Stakeholders will take place on November 28th in Brussels and will be on 'Use and measurement of extended half-life coagulation factor concentrates and novel non-replacement therapies." Registrations will open on October 2nd.

United in Diversity: EHC Leadership Conference 2017

The third EHC Leadership Conference was held from June 29th to July 2nd in Brussels, Belgium. It once again brought together established leaders, staff and young volunteers of various EHC National Member Organisations (NMO) for an exciting event full of activities. The different backgrounds and experiences of the participants made for a diverse group that eagerly shared ideas around a common goal – providing the best possible support to people with haemophilia and their families.

Poster Display

A new element of the programme - poster sessions - was one of the many opportunities that allowed for such an exchange of best-practices. NMOs created poster displays of and presented initiatives they have organised in their respective countries, such as "Starting a conversation with people with mild haemophilia" and "Building a strategic plan" (French NMO), "Building awareness of psychosocial aspects of haemophilia" (Serbian NMO), "Haemophilia and Ageing" (UK NMO), "Applying informed consent to haemophilia treatment" (Portuguese NMO), "Haemophilia and life as a couple" (Romanian NMO) and "Interaction of the Russian Haemophilia Society with other patients' societies" (Russian NMO).

The programme also included thematic sessions on ***gene therapy*** *(presented by Professor Flora Peyvandi),* ***women and bleeding disorders*** *(presented by members of the EHC Women's Working*

*Group), extended half-life products (presented by Dr Beatrice Nolan) and **fundraising** (training by external facilitator Simona Biancu).*

The EHC provided live Russian interpretation throughout the duration of the conference for members of the Russian-speaking NMOs.

As diverse as the group was, so were the ideas each person took away from the conference. Four of the participants share their experience and impressions of the event.

Peer-to-peer sessions were held on the following topics:

- Working with the pharmaceutical companies in time of new products coming into the market
- Healthy working methods between staff & volunteers
- Youth involvement in the NMO – how to evolve
- Inter-generational dialogue in the haemophilia community and succession planning
- Improving the quality of care, including how to build a relationship with a healthcare provider
- Using the EDQM/Kreuth III/IV recommendations in patient advocacy

Claire presented on "Building a strategic plan" during one of the poster sessions

Claire Arcé, Communications Officer at the Association Française des Hémophiles (AFH), EHC French NMO:

This past June, I participated in the EHC Leadership Conference that took place in Brussels, Belgium. I came back to work the following Monday with a pumped-up motivation, a lot of new ideas and a sense of belonging to something great, much bigger than just me and my daily, sometimes tedious, tasks. It reminded me that people with a bleeding disorder constitute a strong community: they are involved, they are committed, they are also a lot of fun and participating in these events is always fruitful.

One idea that I had and that I will try to bring to life is an awareness campaign regarding women with a bleeding disorder. At the French Haemophilia Association, we currently have a quiz that helps women determine if they have a bleeding disorder. I was thinking that maybe we can get a feminine products company on board to display and distribute this quiz on their product boxes. There are still many details that need to be figured out before even contacting such a company, but I am inspired to try.

I'm looking forward to my next participation!

Liora Tevet, Director of the Israeli Haemophilia Association, EHC Israeli NMO:

The Israeli Haemophilia Association was happy to return to the EHC and take part in one of its events! Three of our members, myself included, attended the latest Leadership Conference, each of us holding different positions within our organisation. (Liora was joined by Gur Melamed, Chairman of the Association and Muran Ofek, member).

The various workshops provided relevant information for each of us according to our needs and functions within the organisation. The knowledge we received from the exchange of experiences with other NMOs contributed a great deal in further developing our plans for improving the treatment in Israel and developing the annual program of activities that voice the challenges of our haemophilia community.

Liora and Gur arriving at the Leadership Conference

As a result of this conference, we also decided to establish a local leadership program in Israel, in order to transfer knowledge, skills and empowerment to young people who will develop a sense of responsibility and belonging in relation to the rest of the community.

Alexander May, member and volunteer at Bløderforening, EHC Danish NMO:

Alexander is a participant from the Danish Haemophilia Society

Participating in the EHC Leadership Conference gave me great new insights into various topics, such as challenges in the eastern European countries, the current state of genetic treatment and prolonged half-life products, as well as the chance to socialise. A key insight for me personally was the impact that upcoming novel treatments will have on the future role of the NMOs. Having a group of haemophiliacs, in which a substantial part of the severely affected might effectively become moderate due to new treatment possibilities, will most likely change the services the NMO needs to provide. Paradoxically, expanding the membership base and retaining the existing one might be more difficult, if the treatment improves. However, NMOs will still have a vital role in the fight for increased access to treatment, building a bridge between patients and the treatment services, as well as providing help with the arising challenges of ageing haemophiliacs. Western European countries might be affected first, as several eastern European countries are still fighting to get adequate prophylactic treatment. Speaking of access to treatment, it was great to see that specific initiatives, such as the PARTNERS programme (see pg. 18), are being undertaken by the EHC to tackle this problem.

Marija Nakeska, long-standing volunteer at Hemolog, EHC Macedonian NMO:

When you are part of a small community, such as the bleeding disorders community, every meeting is a joyful experience. You have the time and chance to exchange thoughts, problems and successes with people who face the same challenges as you.

I had attended the very first EHC Leadership Conference, which took place in Belgrade, Serbia, in 2015. True to its name, it was a delightful event that focused on developing leadership skills – qualities, which are important to have in order to move your NMO forward, to be able to advocate and generally, in life. Each conference since has provided a place where the EHC and other societies can share their tools of success in certain fields. Not always can everything be implemented in all countries, but there are a variety of lectures that meet every NMO's needs.

The last Leadership Conference was once again enlightening for me. First, I could easily notice how the event has developed and grown in two short years, covering more topics and with an increased number of people attending. There were various presentations that were very eye catching but for me, something completely new was the one on how gene therapy works. I now understand its concept and it doesn't seem like science fiction or nuclear physics anymore! I can easily share the knowledge with our members without being confused about what it is. Another concept I learned is the different ways to fundraise as an organisation. Many of the suggested ideas are something that we will try to implement in the near future, at least to a certain degree.

Marija (right) is member of the EHC Women's Working Group

I will finish with a thought from one of the greatest football players, Pelé: "Success is no accident. It is hard work, perseverance, learning, studying, sacrifice and most of all, love of what you are doing or learning to do."

The EHC Leadership Conference was made possible thanks to educational grants from Pfizer, Roche, Shire and Sobi.

Update: EHC PARTNERS programme on increasing access to treatment

By Declan Noone, EHC Project Consultant

Since early 2016, the EHC has being striving to develop a concept that pushes the boundaries of their mission to support National Member Organisations (NMOs) and improve the quality of life of people with bleeding disorders throughout Europe. In many countries, the health care systems are often well developed but have limited scope for increases in the purchase of medicinal products. In terms of haemophilia, whilst this budget constraint is faced by every country's own national health policies, some countries are starting from a much lower base and therefore, struggle to provide a basic level of care. To put this in context, the national allotted amount for a child (<18) with haemophilia in the Kyrgyz Republic for the year is equivalent to what an infant in western European countries might receive in a month, and for older children, in just over a week of prophylaxis. The Kyrgyz NMO and clinicians have worked together extremely hard and have been very effective with limited resources, succesfully increasing the amount of product available over the last number of years. They should be lauded for their stoic efforts, as should many other countries in similar situations. Any programme of the EHC and its community should aim to help build on this work and support the local organisations and their respective health care systems in continuing the increase in the availability of factor concentrates in a sustainable way.

EHC President Brian O'Mahony and project consultant Declan Noone visit the Kyrgyz Republic to discuss implementation of PARTNERS

Meeting between the EHC and the Kyrgyz Republic Health Ministry officials, the Insurance fund, haemophilia doctors and members of the Kyrgyz NMO

It is to this end that the EHC has moulded a project under the name of the Procurement of Affordable Replacement Therapies – Network of European Relevant Stakeholders programme, or simply PARTNERS. This programme is a new and innovative approach to creating a sustainable procurement model of

treatment products for haemophilia A and haemophilia B in a set number of countries that currently do not meet the Council of Europe's European Directorate for the Quality of Medicines and Healthcare (EDQM) recommendations[1,2]. The three main criteria required for country inclusion in the programme are for the current use of FVIII replacement therapy to be less than 4 IU per capita, and/or current use of FIX replacement to be less than 0.5 per capita (Kreuth IV: 2017), and the availability of prophylactic treatment for all children with severe haemophilia (Kreuth IV; 2017). The recommended levels of treatment should allow for all children to be on some form of prophylaxis, all adults to be on at least on-demand treatment with home therapy, no restriction on required surgeries and possibly, access to Immune Tolerance Therapy (ITI) for patients with inhibitors. The second component of the programme is how exactly it will work. For this the EHC built on its previous experience[3] and another recommendation from the Kreuth IV EDQM that states "National or regional tenders for factor concentrates are encouraged and should always include clinicians and national patient representatives."

At the annual General Assembly of the EHC in 2016 in Stavanger, Norway, the concept of the project was put to the NMO and there was a resounding commitment from all that this was a project that the EHC should be carrying out. Invitations to discuss the project were sent to all pharmaceutical companies to respond to this call of action from the NMO and meetings took place over the ensuing months to discuss the key components of the program and potential involvement.

The programme has a relatively simple mode of action. If the local health authorities agree to the criteria outlined below, the pharmaceutical companies committed to the programme would bid below a maximum ceiling price for the factor treatment:

- A centralised tender and procurement process with formal patient and clinician involvement
- Willing to award a 3-year contract, and
- Increase factor purchase without decreasing the national haemophilia budget and agree, in principle, to at least double the currently purchased amount of factor replacement therapies over the three-year duration of awarded contracts or until the minimum IU/per capita set by the EDQM is met at any given time.

Based on previously sent surveys[4,5], a total of 14 EHC member countries were eligible for the programme and in February 2017, a meeting was held with ten of them in Paris, France. Each country representative presented their current system of purchasing the factor replacement therapies and the individual challenges they face nationally that could affect a programme like PARTNERS. Countries who were unable to attend were contacted separately.

During the EHC World Haemophilia Day event, three pharmaceutical companies – Kedrion Biopharma, Pfizer and Sobi – answered the call to action and signed a letter of intent to commit to the programme. To date, Ukraine, Serbia and the Kyrgyz Republic have been visited for initial discussions about the programme with the Ministries of Health, payers, clinicians and patients. A further five are due to be visited before the next EHC Conference in October

and all countries who are eligible and interested should be visited by the end of Quarter one of 2018.

The programme has gained strength and momentum in the last few months, and the EHC is hopeful that this will continue and that the programme will start to see real change in the participating countries throughout the coming months and years.

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4. O'Mahony B, Noone D, Giangrande PLF, Prihodova L. Haemophilia care in Europe - a survey of 35 countries. *Haemophilia*. 2013;19(4). doi:10.1111/hae.12125.

5. World Federation of Hemophilia. *Report on the Annual Global Survey 2014*.; 2015. http://www1.wfh.org/publications/files/pdf-1627.pdf.

Update: The EHC Women's Working Group

By Naja Skouw-Rasmussen, EHC Steering Committee member and member of the EHC Women's Working Group

This summer, the EHC Women's Working Group joined the greater conversation by taking part in the UK Haemophilia Societys' Talking Red Conference and the EHC Leadership Conference. Here is a bit of our experience.

Talking Red is the UK Haemophilia Society's campaign to get women talking about bleeding disorders and to create awareness about bleeding disorders in the female community. To this end, this past June, the Society hosted a conference for women with a bleeding disorder, a carrier or their partner. On the agenda was the establishment of support groups at the universities, discussions on how and what kind of support you can get from the social workers and a Q&A session with a doctor about bleeding disorders in women. From the presentations and the following discussions, it was evident that women with a bleeding disorder face difficulty in accessing the correct treatment. One example of this challenge is the facts that they are not offered treatment options or affiliation to a haemophilia treatment centre.

Another very interesting debate was concerning the terminology used for people with a bleeding disorder. An example given was of two individuals, both with a factor level of 20 per cent, both experiencing joint and muscular bleeds and both with prolonged bleeding after operations and trauma. The only difference is that one is female and the other male, hence their diagnosis as a carrier and a mild haemophiliac, respectively. No one could answer why things stand this way but it is a very valid discussion to have, as it can determine which treatment option is right for you.

The EHC Women's Working Group presented on Women and Bleeding Disorders during the EHC Leadership Conference

Next up was the EHC Leadership Conference – a three-day training session for NMO leaders, leaders-to-be and volunteers. For this event, the EHC Women's Working Group planned a workshop on the topic of women with bleeding disorders. The overall idea was to collaboratively explore some of the difficulties women with a bleeding disorder experience and collectively come up with some initiatives on how the NMOs can accommodate these issues. An example of a challenge brought up is that many women with a bleeding disorder neither know other women in a similar situation nor have much

information about their disorder. An idea for how to change this was to establish online support groups, including some educational webinars. By the end of the conference, several NMOs were keen to either establish a committee on women with a bleeding disorder or initiate activities in their support. So dear reader, keep an eye out for information about it within your NMO, or ask them what they are planning to do.

The EHC Women's Working Group will be present and have a booth at the EHC Conference 2017 in Vilnius. Come visit and talk with us!

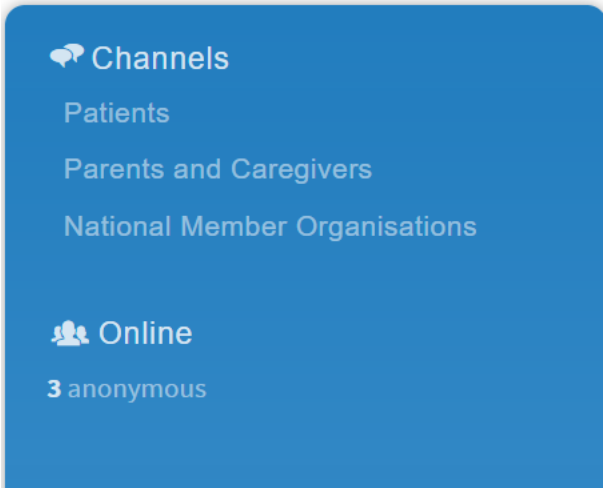
In 2015, the European Haemophilia Consortium (EHC) established the European Inhibitor Network (EIN) programme in order to better support people with haemophilia and inhibitors across Europe. After launching a survey to accurately identify the needs of those living with an inhibitor, the EIN developed several programme elements to target the identified needs. One aim was to create an online platform that will provide a space for PWI to seek information and resources, discuss and share their daily challenges and connect with other peers. And so we did! The website is further divided into sections that are specifically tailored to parents and caregivers, as well as to national patient organisations, with ideas on activities they can implement within their own inhibitor communities for better support and inclusion.

Mirko Jokic, member of the EHC Inhibitor Working Group, introduces some of the concepts the platform will offer. We are excited to officially launch it at the EHC Conference in October (see pg. 53) and invite you to join us at www.ehc.eu/community*!*

1. How was the idea formed to have a website and chat forum specifically for people with inhibitors?

When developing the EHC EIN programme, one of the challenges we wanted to address is the isolation of PWI by establishing an active online community for them.

PWI are a very isolated group of patients due to a variety of reasons, including lack of information inside national societies on the topic and no developed connection between patients and in some cases medical professionals across Europe. As it's the era of digital communication, we should be using these advantages to bring PWI together and provide them with relevant information, news and possibilities to communicate between themselves.



This hub seems to be one of the best way to keep in touch on a regular basis with other PWI if we keep in mind the reduced mobility many PWI experience.

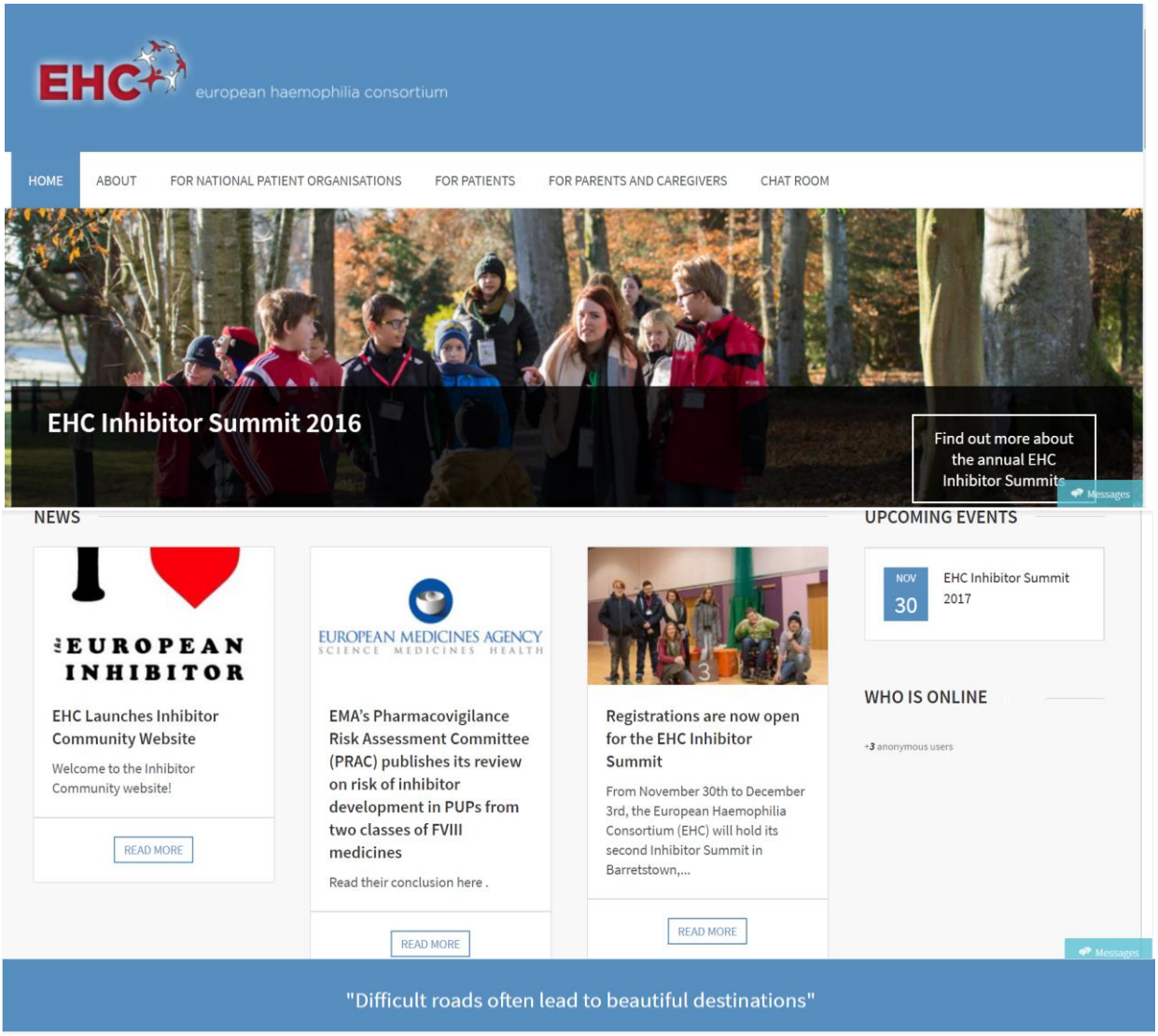
2. What can visitors expect to find?

This website is a combination of an online inhibitor library and a virtual community, making it one of a kind. Visitors can find a lot of useful information about inhibitors, advice on day-to-day issues, travel and professional guidelines, and of course, speak with other peers facing similar challenges.

Also, visitors can have a look at personal testimonies! Find out how other people live, how they deal with inhibitors, and how they overcome challenges.

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

www.ehc.eu/community



NVHP: SUPPORT SYSTEM OF THE DUTCH HAEMOPHILIA COMMUNITY

By Lynnda Pekel, board member of the Nederlandse Vereniging van Hemofilie-Patiënten (NVHP) and Joyce van Steenbergen, volunteer at NVHP, EHC Dutch NMO

The Dutch Haemophilia Society (NVHP) is an association for patients with haemophilia and other bleeding disorders in the Netherlands. With this article, we would like to introduce you to some of the services and activities we offer in efforts to best support our members.

One way of support that has proved effective is our phone hotline, on which patients can call and receive advice on a wide variety of topics from the volunteers operating it. Sometimes, they just lend an ear, which is just as important.

We also organise several gatherings, at which we provide relevant information and which serve as a platform for mutual contact among patients. Below are descriptions of some of these activities.

Our biannual member meetings are always worthwhile. Besides focusing on the typical topics that arise in a member association, we also include a half-day session that examines a specific concern in detail. The lunch and informal drinks that follow give an opportunity for everybody to catch up and socialise. We offer child care services so that parents can fully benefit from the meeting and informative sessions. Some kids enjoy it so much, they eagerly ask their parents when the next meeting will be!

Once a year, we organise a weekend for families with young children (0-10) on a campsite in the middle of the country. We reserve a large area with tents for meals, activities and for

people to stay in, if they haven't brought their own. The most desired outcome is to have a great time with people to whom you do not have to explain why you are so careful with your child and who recognise the troubles, challenges and successes. Young children (and their parents) see how growing up with haemophilia can be a "normal" thing. We have also had expecting couples attend in preparation for the birth of their child with a bleeding disorder.

Although these weekends were originally conceived for families with young children, in the last few years we have also started to provide a separate tent for teens, in order to include them and give them a place to hang out together. This year, they went on a late-night laser game expedition, which was a lot of fun.

Throughout the two days, we have young adults there to provide support and assistance with the kids. This integration of the different age groups not only ensures parents that their kids are well cared for but also prepares the youth in knowing how to organise such events in the future (next generation of leaders!).

To kick off the weekend, we have some fixed activities, such as an icebreaker game, a barbecue and an open mike night. There is always a theme this year, it was fairy tales and magic – a great topic that elicited a lot of wonderful contributions. In line with that concept, there was also a magician and a life-size board game.

One of the activities with a great value is where teens and young adults show the younger kids how they infuse themselves with factor treatment. The children see that they are not the only ones that have to go through this and are usually very impressed with this knowledge and experience for years to come! It also gives parents the opportunity to exchange "tips and tricks."

Last, we always have an invited expert speaker – this can be a doctor, social worker, nurse or physiotherapist. The presentations are organised separately for parents and kids, giving everybody space and a peer group of their own age and experience.

This year for the first time, we organised a special day for women and girls with bleeding disorders. Patients and carriers came together to listen to experts and discuss topics, such as menstruation and pregnancy. It became clear that there is still a lot of denial about women and bleedings disorders, as even some doctors still tell women that only men can suffer from a bleeding disorder. There is an obvious need for information and effort to give these women a voice and provide them with the right diagnosis and treatment options.

This is only a small sampling of our activities! If you want to know more, please contact us, we'd be happy to share our experiences and lessons learnt! And of course, we welcome your ideas that can benefit our members and their families.

Amendment of German HIV-foundation-law for victims of the blood-scandal finally achieved

By Dr Anna Griesheimer from the Deutsche Hämophilie Gesellschaft (DHG), EHC German NMO

It has been a long and difficult struggle which finally found a fortunate end: for many years, the German Haemophilia Society (DHG) had been fighting for an amendment of the HIV-foundation law. The aim was to guarantee lifelong compensation for the victims of the blood scandal. On July 2nd, 2017, the corresponding amendment was passed by the German Parliament! This is our story.

The German HIV-foundation for the victims of the blood scandal, *Stiftung Humanitäre Hilfe für durch Blutprodukte HIV-infizierte Personen*, was founded in 1995. Since then, the beneficiaries – people who were infected with HIV by contaminated blood products in the 70's and 80's – have received monthly payments from the foundation. The donors were the State, six pharmaceutical companies and the German Red Cross. However, there was a critical paragraph in the law, which stated that the foundation would be liquidated as soon as the purpose of the foundation was fulfilled *or* ***the financial resources were exhausted***. Thanks to modern therapies, it became a "problem" that the life expectancy of the beneficiaries had enormously increased. Several times, the foundation ran short of money and its existence was threatened. Every time the DHG stood up for its continuation. Each time, additional donations ensured the foundation's survival at the last moment, but only for a limited time frame.

When the topic arose again at the beginning of last year, it had become almost a routine to approach the "donors" with the request for further donations. But this time, the DHG Board, under the then-chairman Werner Kalnins, did not want to accept that, once again, this would be a solution for only a few years. What the affected people urgently needed was long-term security! The numerous worried calls of highly alarmed beneficiaries, anxious about their future, encouraged the Board to initiate further actions.

In the summer issue of our membership magazine, we asked our members to speak up and address the deputies of their constituency with a letter, in order to put pressure on the policy-makers in Berlin. We provided a sample letter and were astonished that so many people responded to our call. The ball started rolling...!

Already in September, it appeared that the topic had become a subject of discussion in Berlin. A Member of Parliament raised the issue of the foundation in a speech before the Parliament, and the party DIE LINKE submitted an inquiry to the government. In the following months, we had numerous dates with deputies of different parties, among them the respective spokesmen for health policy.

In October, on the eve of our General Assembly in Fulda, we organised a meeting for the beneficiaries of the foundation. Many of them had accepted our invitation and a fruitful discussion about our further activities arose. Also at the General Assembly, a new DHG Board was elected. Chaired by Dr. Stefanie Oestreicher, the new Board decisively continued the struggle for the amendment of the law. Letters to chancellor Merkel and vice-chancellor

Gabriel, as well as many intensive discussions with other relevant politicians, allowed us to move forward step by step.

How great was the relief and joy when in the middle of March, we got several hints from Berlin that indicated that indeed, an amendment of the law was planned. Soon, it turned out to be true: the governing parties had proposed a law change that included the guarantee of lifelong payments, as well as an adjustment of the payments according to the increase of the pensions in the future. From 2019 onwards, the State was intended to be the only donor.

On 26th April, the DHG was invited to a public hearing of the Health Committee of the Parliament. Björn Drebing, our deputy chairman, made clear to the committee that we welcomed the proposed amendment of the law, but that we still regard the pharmaceutical companies as morally responsible for the financing of the foundation and that we also consider a retroactive inflation compensation as necessary

The amendment of the law was passed by the Parliament on June 2nd, unfortunately, not with all the changes we had proposed. Nevertheless, we are very, very happy that the beneficiaries finally got the guarantee for lifelong payments and that these payments will be adjusted in the future. Full of joy we can say that our persistent efforts paid off. We finally have achieved a great goal and it is worth fighting…

EHC Activity Grant

Last year, in support of its National Member Organisations (NMOs) the EHC created an activity grant to help with co-funding of NMO activities that aim to improve the quality of life of people with haemophilia or other bleeding disorders. The grant programme was designed to help NMOs in situations where a lack of national funds would have otherwise impeded their work towards the benefit of their patient community. EHC Activity Grants help co-fund new or existing areas of work in a short-term and ad-hoc fashion until sustainable funding can be secured by the NMO.

One of the recipients this year, the Ukrainian Haemophilia Association, used the funding to organise a summer camp for children with haemophilia and their families. The following is a story in pictures of the adventures and emotions of the kids at the 'Forest Glade' camp, which the association hopes to make an annual event.

**The article is written by Viktor Kronikh, Board member of the Всеукраїнське товариство Гемофілії, EHC Ukrainian NMO*

From June 15th-20th, the Ukrainian Haemophilia Association held a summer camp for children with haemophilia and their families in the picturesque and full of wildlife Ukrainian Carpathian Mountains. 14 kids between the age of 8-17 years joined for a true camping experience – they stayed on a campground in the woods, cooked food by the fire and took part in outdoor activities.

The early wake-up calls didn't take away from the energy level and the kids spend the days playing football and other sports, as well as participating in intellectual and creative games. To assure their complete safety, prophylactic treatment was provided and health care workers were available for any kind of assistance throughout the duration of the camp. The children are a great example that with timely preventative treatment, people with haemophilia can lead a healthy and active life.

The campers stayed in wooden huts, known as Hutsul kolybas. According to camp rules, parents stay offsite at a hotel in a near-by village.

Always under the supervision of experienced volunteers, the campers stayed quite busy – from artistic and scientific masterclasses, group performances and henna drawings to

exploring interesting areas around, there was always something to do! Of course, there were many surprises as well.

On one of the mornings, the children were awaken by an unusual alarm clock – the sound of a trembita. The trembita is a Ukrainian alpine horn made out of wood and is considered to be the longest musical instrument in the world. The campers were introduced to its sound and history by a famous trembita player and had the opportunity to try the instrument themselves – something they did with great enthusiasm.

Campers took turns playing the trembita

Another memorable experience was the visit of the regional organisation “Plast”, the largest Ukrainian scouting organisation. It’s members, not much different in age from the camp participants, shared their knowledge on survival in the wild and taught the kids how to tie complex knots, start a fire, search for water sources and protect themselves against harsh weather conditions.

To further develop their survival skills, campers learned to navigate the locality with the help of a map and a compass, as well as without them. In doing so, children and adults made their way through a mountain stream by a rope zip line. Though it might look a bit extreme from the side, everyone was very safe in the hands of experienced zip liners.

Other activities included:

A pottery workshop by a well-known local potter and art therapist.

A mountain trip through the 'Valley of the Forest' for both the children and their parents. Their tireless hiking was rewarded with a lunch that included local cheese dishes and a panoramic view of the breathtaking landscape.

Therapeutic baths with healing tea brewed from Carpathian herbs.

The camp came to an end too fast but created great memories and stories to last until the next time around. The Ukrainian Haemophilia Association was happy to receive feedback from the parents, who reported that their children had become more independent and self-confident.

The Ukrainian Haemophilia Association thanks the Lithuanian Haemophilia Society and all the volunteers for their invaluable support and direct involvement in the organisation of the "Forest Glade" camp.

We also want to express our gratitude for receiving the fund from the EHC grant program, which contributed greatly in making this event possible! This was the first time that we have applied for grant funding and our friends from the EHC guided us through the whole process of preparation and application. We are happy that this event was successful; it is our hope to make it an annual activity and to be able to extend it to youth participants from neighboring countries.

Marking of World Hepatitis Day in Azerbaijan

By Gulnara Huseynova, Chairman of the Hemofiliyalı Xəstələrin Respublika Assosiasiyası, EHC Azerbaijan NMO

On July 28, the Azerbaijan Association of Patients with Haemophilia joined the Association of Gastroenterologists and Hepatologists for an event in Baku to mark World Hepatitis Day.

HCV information brouchers were passed to raise awareness

Though viral hepatitis affects millions of people across Europe, it often shows no symptoms and a large percentage of cases go undiagnosed. The occasion was an opportunity to inform the public and raise awareness around this "silent disease" and the consequences it carries. The Chairman of the Association of Gastroenterologists and Hepatologists of Azerbaijan, Dr Gulnara Aghayeva, shares that the months of preparations and negotiations with sponsors and the management of the event venue were well worth the effort to spread an important message.

"We really wanted it to be successful and reach as many people as we could. We distributed hundreds of information booklets and vouchers for free tests for viral hepatitis! – Dr Aghayeva

According to the Ministry of Health, at present there are 1,541 people with moderate and severe forms of haemophilia in Azerbaijan. Close to 70 per cent of patients aged 20 years and older are infected with hepatitis C (HCV). Fortunately, since 2006, due to the availability of high-quality haemophilia treatment and improvements in plasma and blood screening, there have been no new cases of infection within the country's haemophilia community.

Members of the Association of Patients with Haemophilia during the event

Still, diagnosis and access to HCV treatment remain a challenge and members of our Association of Patients with Haemophilia are working to change the situation.

We, as well as everyone else who has been affected, hope for modern, non-interferon therapy with direct antiviral drugs. Right now, because of the high price, this medication is not available to most of the patients. In response, our organisation is currently preparing appeals to state structures, which call for patients with haemophilia and HCV to be provided with treatment. We hope that through advocating and putting pressure on politicians, the government will more quickly adapt a national HCV eradication plan.

The Azerbaijan Association of Patients with Haemophilia celebrates 20 years since its establishment

By Gulnara Huseynova, Chairman of the Hemofiliyalı Xəstələrin Respublika Assosiasiyası, EHC Azerbaijan NMO

Exactly 20 years ago in 1997, the outlook for people with haemophilia in Azerbaijan was very grim – there was a shortage of treatment, patients suffered from frequent bleeds and were left with life-long disabilities.

As I have a son with haemophilia, I wanted to unite parents and patients so to address these challenges together. This idea gave birth to the Azerbaijan Association of Patients with Haemophilia!

In recent years, we have witnesses a completely different scenario. Now, there are programmes to educate families on haemophilia and the overall care in the country has greatly improved. The attitude and approach of health care physicians to haemophilia has changed for the better and prophylactic treatment has enabled children with haemophilia to grow up health, be able to attend school and lead a quality life.

In honour of the 20th anniversary of our association and in order to continue our contribution to the improvements in haemophilia care, our members initiated a new programme called "Mother and Child". In its framework, we organise training sessions for parents on how to deal with different aspects of haemophilia.

The last such workshop was directed towards parents of children with severe haemophilia. In his welcoming speech to participants, the Executive Director of our association and Chairman of the Azerbaijan Union of Patient Organisations, Ayaz Huseynov, emphasised on the importance of prophylactic treatment and access to home treatment.

Training on administration of intravenous injections

Mothers of children with haemophilia who participated in the workshop had the opportunity to learn how to administer intravenous injections by practicing on a dummy. There were also various presentations on topics ranging from maintaining good oral hygiene to the correct usage of elastic bandages on children with haemophilia.

There were separate activities organised for the young participants

We are proud to celebrate 20 years of the Azerbaijan Association of Patients with Haemophilia and will continue to advocate and initiate projects that support our haemophilia community!

Participants in the "Mother and Child" training workshop

Mr Joško and Acquired Haemophilia

By Dragica Bošnjak, member of the Društvo hemofilikov Slovenije, EHC Slovenian NMO; Translation by Vera Andjelković

Almost on the outskirts of Ljubljana, Slovenia and down a long stretch of the Ižanka road, you enter a completely calm, beautifully landscaped rural setting. Just a stones' throw away from the attractive Iška Gorge lies a homestead, where one can experience pristine contact with a way of life opposite to the everyday chaos of the city. In this quietude, you do not notice when and how time passes by.

It is here that you are greeted by Jože Škulj.

Known to everyone as Joško, he is a man of many skills – an auto mechanic, farmer, horse breeder and engrossing storyteller. Between him, his vibrant wife Danica and family members of their two succeeding generations, there are enough stories to keep you occupied with for hours. The large stack of photo albums and various trophies inside the family house testify to their interesting way of life. Many are the awards brought home from the winners podium as recognition of hard work in a variety of sports and other activities by all the generations – from the youngest to the eldest.

The whole family is involved in the long tradition of horse breeding while the rest of the responsibilities are divided up between the generations – the farm and the car repair workshop have long been handed over to Joško's two sons, Boštjan and Andrej.

Joško, his wife Danica and their granddaughter Maša

Jože Škulj, born in 1948, has lived in the area for 66 years. He was born on the outskirts of the Iška Vas village but later on his family relocated closer to its centre, where they built a house and made their home. Given his familiarity with the area, Joško can share many interesting curiosities about the village, the nearby marshland and the surrounding towns, where they say you can taste the cleanest drinking water in the country. According to Joško, the uncontaminated water is "very suitable for making spritzers or raspberry syrup."

The near-by Iška stream has the same reputation – locals proudly tell of how industrial pollution has not yet made its way to the stream and that it remains a favorite summer spot.

Joško knows how to appreciate the area. He has contributed significantly to preserving local traditions, such as farming and harness horse racing. But you can also catch him reminiscing on his early years, when he trained to be a auto repairmen and made his living at the mechanic workshop in Ljubljana. In those days, the process of repairing cars was very different – spare parts were rarely available, there were no lifts or auto body alignment benches and tools were at their most basic. Joško had to spend a considerable amount of time underneath the cars lying on concrete, which, as he says, "undoubtedly added to the development of rheumatism later on." Still, he opened his own shop and grew the business until his younger son, Andrej, took over, while Boštjan dedicated himself to farming.

Though each member has responsibilities of their own, the whole family loves being involved with horse breeding. For the purpose, they use the Slovenian cold-blooded horse – a mighty animal that can reach anywhere from 700 to 1000 kg, while at the house they also have ponies and riding horses for the children. Altogether, the stables on the farm are home to 35 horses, with 12 to 15 foals being born annually.

Joško with some of the family's beloved horses

This all began with one mare somewhere around 1978. Joško's parents were also owners, but starting in the sixties, horses on most farms gradually began to be "replaced" by tractors and other machinery. That was not the case for the Škulj family, who around half a decade ago decided to dedicate themselves to horse breeding. Until last year, Joško was also the President of the Breeders Association of the Slovenian Cold-Blooded Horse. The love for horses has been passed down all to way to Joško's granddaughter, Maša, who confidently rides even the biggest horses on the farm.

Life on the Škulj homestead has always been dynamic. Though Joško's wife Danica is a pensioner, on a farm, that represents little change – most make every effort to be active as long as they can. Even when surprised by the development of a medical condition…

It is this sort of an unpleasant surprise that Joško received when doctors discovered he had acquired haemophilia; a surprise because this type of a bleeding disorder is extremely rare and as the name implies, is not congenital but can develop at any time in life. After hurting his leg from falling down, Joško went to the hospital due to fluid accumulation in his knee. While draining the excess fluid, doctors noticed large amounts of blood mixed in with the fluid and couldn't get the bleeding to stop. Blood continued to accumulate inside the joint, while the knee was constantly bleeding on the outside.

Doctors questioned Joško on his previous surgeries and whether he had had similar problems with bleeding for long periods of time. He assured them that neither during his two previous smaller operations, nor at the dentist's office, did he experience such problems. He had also cut himself countless times while working at the auto shop, but had never experienced serious bleeding.

Once they finally reached a diagnosis, Joško began his factor therapy treatment. He didn't concentrate on how or why this happened to him, but rather on following the medical advice on how to best manage it. Both him and Danica are now very well informed about the disorder and have returned to the rhythm of life on the farm, where thoughts of the past bring a feeling of accomplishment and looking into the future is as exciting as ever.

Tracing the Treasure Chest – The Haemophilia Camp of Turkey

By Demet Mete, Board Member and Secretary of Türkiye Hemofili Derneği, EHC Turkish NMO

The Haemophilia Society of Turkey has annually organised summer camps that bring together people with haemophilia from all over the country. The 23rd Haemophilia Camp was held in the Drazali Recreational Facility in Bursa from July 17th - 21st and was attended by 146 people with haemophilia and their families, doctors, nurses, a psychologist and sports instructors.

The theme of this year's camp was **"Tracing the Treasure Chest"**, which represents the "chasing" of solutions to everyday life challenges, with the most important concept being health.

The program began with a welcoming speech that informed participants of the activity options they have – from sports in the morning to entertainment for children and informative treatment sessions, there was something for everybody. As swimming is a great sport for haemophiliacs and has a positive impact on the physical development and strengthening of joints, water therapy sessions and swimming lessons were offered every day for three hours.

On the second day of the camp, participants were taken on an "Iznik Culture Trip" and had the chance to visit the museum of Aysafya, the door of Lefke and the hill of Abdulvahap while a tour guide explained the history of these sites. The day ended with a singing competition "The Voice of Haemophilia", in which three adults and four children made it to the final round. The winner wasn't announced until the closing ceremony on the last day of the event.

The scientific portion of the camp program included presentations on treatment of orthopaedic problems in people with haemophilia, the importance of dental hygiene, classification of factor levels, inhibitor development and gene therapy. The President of the Haemophilia Society of Turkey Dr Bulent Zulfikar also gave a detailed update on the recent developments in haemophilia care.

A special part of the programme was the projection of a video that took participants on a journey through the 25-year history of the society, whose mission has always been to serve and provide solutions to the challenges of people with haemophilia.

We are very much looking forward to meeting old and new campers during next year's haemophilia camp!

The New Danish Times

Interview with Jacob Bech Andersen, President of Bløderforening, EHC Danish NMO. Interview by Raia Mihaylova, EHC Communications Officer

In early May, after 32 years of leadership in both the organisation and within the haemophilia community, Terkel Andersen stepped down as President of the Danish Haemophilia Society (Bløderforening). In his farewell speech, Terkel shared that he feels this as the right time to resign and does so with great confidence in the younger forces that are ready and driven to move the association forward.

And driven he is – the new president, Jacob Bech Andersen, has been on the Bløderforening Board of Directors since 2005, has held the position of Vice-President together with Tem Folmand since 2011 and contributes to and edits the Society's magazine. He stands behind various initiatives, such as the network to bridge the gap and establish better communication between those in the age groups of 30 to 49, or the "young old."

By profession, Jacob is a sports journalist. He lives in Solrød together with his wife and two young children. Through this interview, we get to know him in his various roles.

Jacob Bech Andersen, President of the Danish NMO

What is your vision for Bløderforening? In the past you have focused on building a bridge between the younger and older community members, will that continue to be an aim?

JBA: My general aim is to continue the very good job the ex-president has done for the Danish Haemophilia Society. We have a really good and well-organised Society – our office has a staff of three people and we are very proud of our efforts in the Danish haemophilia community. Of course, as you mentioned, I also have a focus on the younger generation. There is a lot of talk that if you are born with haemophilia today, then you don't have any problems, you can get all the treatment you need in Denmark. But our experience is that young people do have problems, especially when they are in their 20s; they can have a lot of challenges and I would really like to help them in dealing with those issues.

We also have the goal to maintain the very good health services we have for people with haemophilia. As one of the rich countries in the world, we have access to the best treatment and it is important to maintain that.

Do you find that the younger generation of haemophilia patients are harder to engage within the Society?

It is a little harder today than it was before, and this is the same all over the world. When you have a generation that leads a life without the big problems of the disorder, we often have to tap them on the shoulder, so to say, and ask if getting involved in the Society is something they would be interested in. But when we have a lot of activities and when we offer all kinds of ways to be engaged in the society, then people come themselves. It is just about finding what is interesting to young people. For example, for myself, because of my interest in communications and media, I was initially asked to help out with our magazine and that was a really good way to get me involved. It's the same way we should approach the young generation now. If you have a young person who is an expert in IT and computers, for example, then you should ask him to help with those kinds of matters, or if you have someone interested in global communication, you can maybe get him to help with a twinning project or something like that.

What is it like to take over a position that the previous president held for 32 years?

It's a huge task because not only was Terkel president for 32 years, he is also pretty famous in Denmark. He is a, what could I call it – "an organisation star" – and he has been doing so many incredible things for the Society. He was a big part of fighting the hard challenges we had in the 80s with HIV and did tiresome advocacy through media campaigns and so on. It is impossible to follow in his footsteps because Terkel is Terkel and Jacob is Jacob. But I think it will be okay because Terkel has enabled the younger generation to be ready to take over. We have a great community with really engaged people who want to make a difference and improve the lives of people with haemophilia. So, I think it will be alright but it will be difficult because he is a big person for the Society.

You have the same last name as Terkel. What else do you have in common?

What we really have in common, which is the most important thing, is that we really, really love this Society and we will do everything we can to keep its good status. There are not that many people like Terkel – he speaks, I think, 12 languages and he had the highest college GPA in the 70s in all of Denmark, so he was the smartest person is Denmark! We share a love for the Society and we are both a little stubborn and don't back down when there is a struggle along the way. We both try not to be that serious at all times, to have a touch of humour with our work, it often helps.

Can you speak a little about the relationship between the Danish Haemophilia Society and the EHC, how do you view it?

It has always been a really good relationship. It started from the very beginning, as Terkel was one of the EHC founders and its first president. We have always turned to the EHC to gain knowledge on the challenges throughout Europe and to get wiser. We have also had members from our Society as members of the EHC Steering Committee, Theis Bacher and now Naja Skouw-Rasmussen, and we always try to participate in EHC events. It has been a really good cooperation for many years and I know it will continue.

You are a journalist. How do you combine that with being President of the Society? Do you have any free time left?

These are two very different topics – I am a sports journalist, I write about sports. But I use my ambition as a journalist in my communication within the Society and maybe that's one of my strongest qualities. I love talking and am good at communicating with a variety of people – young people, older people, politicians, other journalists, etc.

It is time consuming but it is important to mention that yes, I am the new President, but we also have a new board that work really hard and are very committed to the Society, an extremely good Vice-President Tem Folmand, with whom I have been friends for 35 years; he has been Vice-President for many years and has a lot of experience. It's not just me who is going to work a lot, it's the entire team and we want to do that! Of course, as a man at my age of 42 with a family and job, you think about if you have the time to do it all. It will be pretty busy but it's going to be okay and well worth it.

How do you see the future of haemophilia?

Of course, I am looking forward to see how far this development with medicine will go. I really, really hope that within the next ten years, more people around the world will have access to treatment. Maybe it's a little unrealistic goal but I would really like to see my friends from countries not as rich as Denmark to have access to perfect treatment. This environment we are experiencing now with all the novel treatments will affect the countries who are not that well-developed. It is really exciting times, I am looking forward to see what will happen. It is difficult the exact difference these novel medicines will make but I am excited to see what the future holds.

The EHC wishes Jacob much success in his endeavours as the President of the Danish Haemophilia Society!

Feature Articles

Researcher spotlight: Dr Roberta Palla

Interview by Raia Mihaylova, EHC Communications Officer

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

Dr Roberta Palla

Dr Roberta Palla graduated with a degree in Biotechnology in 2002, followed by a PhD in Clinical Methodology from the University of Milan. She is currently a researcher at the Luigi Villa Foundation of Milan and carries out her research at the Angelo Bianchi Bonomi Haemophilia & Thrombosis centre. Her interests include the characterisation of clinical and laboratory (genotype and phenotype) aspects of rare bleeding disorders (RBDs).

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

My research interests include mainly bleeding disorders. Since the very beginning, I have been working on the genotype-phenotype characterisation of rare bleeding disorders (RBDs), but also on the molecular and immunological aspects of patients affected by thrombotic microangiopathies, with focus on thrombotic thrombocytopenic purpura (TTP). During the years, we have collected information on many patients and we were able to implement the International Rare Bleeding Disorders Database (RBDD, www.rbdd.org). This database takes up a lot of my time, but it is very satisfying as we are now able to draw significant conclusions on the clinical picture and management of RBDs. For the last two years, I have abandoned the microangiopathies field in order to dedicate part of my work to haemophilia, particularly to the Survey of Inhibitors in Plasma-Product Exposed Toddlers (SIPPET) study/ project, and therefore to the causes and risk factors for the development of inhibitors.

How did you become involved in this field?

I have been working on bleeding disorders since my training for my graduation thesis in Biotechnology. While at the university, I heard about the research of Prof Flora Peyvandi and Prof Mannuccio Mannucci on bleeding disorders and I became interested in it. Therefore, I asked to do my training with them. After finishing my degree, Prof Peyvandi gave me the possibility to continue my education with her through a PhD degree. So, my life in haemostasis began for many years with a post-doc position and finally, with a permanent position as of last December. Unfortunately, in Italy there are very few positions in research and particularly very little money!!!

What does your average day look like?

Wakeup call is at 6, followed by cuddles with my daughter and taking her to school. I then take the train and subway to go to work (I live in a small town just outside of Milan) and arrive at the Angelo Bianchi Bonomi Haemophilia and Thrombosis centre of Milan. Now, after many years in the lab, I mainly work in front of a computer to analyse data, write projects and papers. Then, there are meetings with lab staff to discuss various study results and to decide how to proceed from there. After closing the door to my office at the end of the day, I am again on the subway and run to my daughter for more cuddles and games. There is also, of course, domestic work, followed by me collapsing in bed!

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

As everybody in the field of medical research, I hope that my research results will impact the lives of patients; make them better and less painful.

What keeps you awake at night?

Nothing keeps me awake at night, I am a very heavy sleeper – maybe only my daughter when she wakes up from a nightmare or for a bathroom trip. Luckily for my mental health, I am capable of switching off my brain when it is time to go to sleep.

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

My/our hypotheses have resulted true until now. I am a very lucky person ☺! There is only one thing that has positively surprised me a lot, I would have never hoped for such a result, but I can tell you nothing more because it is related to my project funded by EAHAD (The European Association for Haemophilia and Allied Disorders) and it is very confidential right now… we are still waiting for the confirmation of the results.

As you briefly mentioned, you were one of two EAHAD research grant recipients this year for "High throughput analysis of antibody binding profiles in previously untreated patients with severe haemophilia A". Can you tell me a little more about this, what exactly it means and what kind of questions the results can potentially answer?

I was very honoured to be awarded such a grant and I am grateful to the scientific committee, which selected my project for funding.

As you probably know, the SIPPET trial demonstrated that patients treated with recombinant FVIII products have a two-time higher risk to develop anti-FVIII inhibitors than those treated with plasma-derived products. In post-translational modification, von Willebrand factor (VWF) content and presence of other proteins have been suggested as plausible mechanisms. However, an explanation for higher immunogenicity of recombinant products requires further investigations. In this project, we are using a new high-throughput technique known as Mimotope Variation Analysis (MVA) to identify the immune profile of patients before and after development of anti-FVIII inhibitors who had not previously been exposed to any blood product, and to find sets of immune-profiles specific for patients treated with a specific

plasma-derived or recombinant FVIII product. The results of this project may help to elucidate the peculiar immunogenicity of each class of FVIII products and to identify patients more prone to developing inhibitors to FVIII after exposure to concentrates.

After many years of little development in terms of treatment and understanding of inhibitors in haemophilia, there now seems to be quite significant advances in the area. What are your thoughts, are we any closer to knowing why they form in some people and not in others?

Incredible results have been achieved in this field in the last four years, and much promising data on the multifactorial mechanism for the inhibitor development was recently shown at the last International Society on Thrombosis and Haemostasis (ISTH) Congress. Nonetheless, I think that the mechanisms underlying this situation are very complex, many pathways are involved, as well as many intrinsic and extrinsic risk factors. The more people who work on this issue, the closer the collaboration among different groups becomes, and we can accomplish a more significant impact of research results.

You are involved in the implementation of an international rare bleeding disorder database. Can you talk some more about it – its aim, development and progress?

Since 1996, the Angelo Bianchi Bonomi Haemophilia and Thrombosis Centre of Milan has been a national and international reference centre for diagnosis and management of patients affected with RBDs. A large and regularly followed-up patient population coming from different countries allowed us to expand our knowledge on the different aspects of RBDs and to form a platform that focuses and develops further diagnostic and therapeutic research activity. In 2004, under the guidance of the International Society of Thrombosis and Haemostasis, Prof Flora Peyvandi and her group, including me, were enabled to spearhead the development of an international network of care providers and national representatives in order to work together to discuss the prevalence, clinical manifestations and need for coordinated and consistent data collection on RBDs. The hope of this international community was and still is to better identify the number of affected individuals throughout the world, define the clinical manifestations and sequelae associated with these disorders, create a network of individuals who care for these patients and are able to share diagnostic and treatment expertise, and identify centres where specific products, once developed, may be utilized in clinical trials.

As a first step, a European project, the EN-RBD, was performed. This project helped to elucidate the association between factor coagulant activity and patients' bleeding severity. Despite the valuable findings obtained, it was recognised that there are still knowledge gaps on the annual incidence of the specific disorders or of the bleeding episodes experienced by patients. These questions were not answered by retrospective data collection and required a prospective evaluation. Therefore, we are now carrying out a project to capture prospective data on patients worldwide with the aim to evaluate the incidence of bleeding episodes, the benefits and complication of current treatment regimens and to find the minimum coagulant activity level required to prevent spontaneous and trauma-related bleeding and to provide adequate haemostasis. Many promising results have been achieved for FXIII deficiency,

recently published in the Journal of Thrombosis and Haemostasis, and we are now focusing on fibrinogen, FV, FV+FVIII and FXI deficiency.

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

Surely the new mimetic products and gene therapy for the treatment of haemophilia. The results achieved so far are incredibly fascinating. For the rest, why unveil the future? As Alan Kay, the computer scientist, said, “the best way to predict the future is to invent it”.

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

When I was a child, my big dream was to become an astrophysic; thus, if I had not been working in haemostasis, I would have been the new Margherita Hack!!!

**Astrophysics is a branch of space science that applies the laws of physics and chemistry to explain the birth, life and death of stars, planets, galaxies and other objects in the universe.*

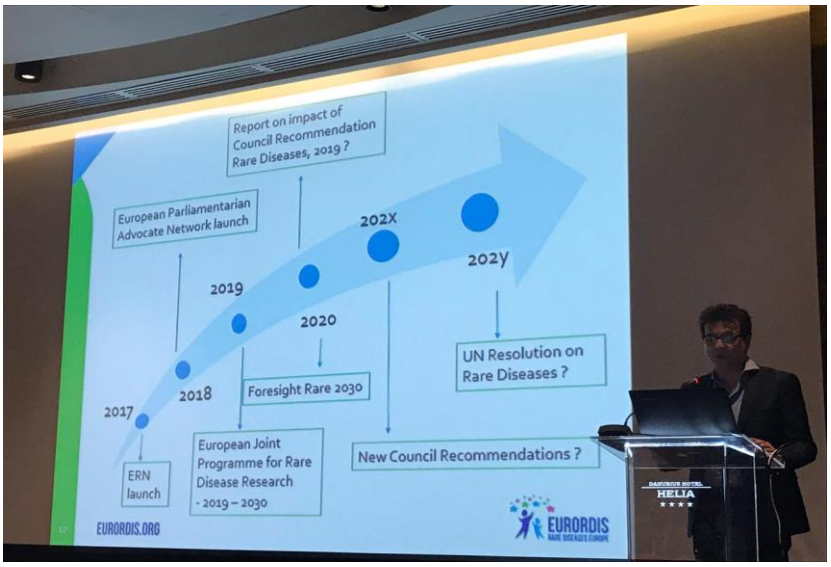
Defining the future of rare diseases

By Laura Savini, EHC Public Policy Officer

During its last membership meeting, held in May in Budapest, Hungary, EURORDIS – Rare Disease Europe, the European organisation representing people with rare diseases, announced its new work plan towards 2020 and beyond. Over the years, EURORDIS has been instrumental in putting rare diseases on the European policy and legislative radar, and EHC representatives Ms Olivia Romero-Lux, Steering Committee member, and Ms Amanda Bok, CEO, learned what lies ahead on their agenda.

The field of rare diseases has come a long way in the last twenty years and yet, much remains to be done! Interesting parallels can be drawn between the evolution in awareness and initiatives to tackle rare diseases and changes within the regulatory and pharmaceutical landscape. Notably in the 1990s, blockbuster medicines, or medicines that focused on treating 'big' diseases, such as diabetes and cardiovascular diseases, were the standard protocol whereas nowadays, medical advances point towards the need for prevention, better diagnosis and personalisation of medicines, treatment and care.

A few key landmarks for rare diseases were the development of the Orphan Medicinal Product Regulation[1] (1999), which for the first time in European legislation gave a definition of what can be considered a rare disease in Europe, i.e. a disease with a prevalence of less than 1 person in 5,000. The legislation prompted the creation of the Committee for Orphan Medicinal Products (COMP) within the European Medicines Agency (EMA), which is in charge of assessing and granting orphan designations. Since



Yann Le Cam, CEO of EURORDIS, presents the roadmap for the future work of the organisation

[1] Regulation (EC) No 141/2000.

its establishment, over 1,800 medicines in Europe received orphan designation; however, only a few (126) were approved, clearly showing the struggles for novel therapies to be developed and to reach patients. Another landmark policy, which pushed rare diseases to the forefront of the European health policy agenda, was the 2008 Commission Communication on Rare Diseases, followed by the 2009 Council Recommendation on an action in the field of rare diseases. These two policies prompted the development of a European strategy to tackle rare diseases, which led to many strong initiatives, such as the development of national plans on rare diseases, initiatives to define and classify rare diseases, the development of centres of expertise and reference networks on rare diseases to promote research. They also displayed the need to empower and involve patient organisations in policy discussions and the decision-making process related to medicine development. In 2011, the European Cross-Border Healthcare Directive[2] reiterated this mission and addressed the need for collaboration in the field of health technology assessments. Unfortunately, the legislation missed the opportunity to ensure that people with rare diseases would be able to be reimbursed for treatment in other European Member States. Nonetheless, all of these actions pointed towards an increased cooperation on a European level, which seems logical when dealing with diseases that only affect very few patients nationally and for which there are limited resources and medical expertise. Finally, earlier this year, the European Commission formalised 24 European Reference Networks (including EuroBloodNet – see EHC May 2017 Newsletter).

Amanda Bok, EHC CEO, presented at the event on patients' expectations and challenges of the ERNs

Throughout all this, EURORDIS has been one of the forces pushing for further recognition of and investment in research, treatment and care of rare diseases. Founded in 1997 thanks to the support of funds from the French AFM Téléthon, EURORDIS had a membership of 187 patient organisations in 2000, while now, it counts amongst its members over 700 patients' organisations, 58 patient federations and 34 national alliances from 32 countries. EURORDIS has been the voice of the rare disease patient community in reaching European institutions, such as the European Commission and the EMA, in initiatives, such as EUnetHTA, and international organisations, such as the United Nations. Furthermore, EURORDIS is also involved in establishing meaningful dialogue with the pharmaceutical industry. Catering to the needs of such an extensive and diverse membership and advocating to such a variety of stakeholders is certainly no easy task and yet, EURORDIS has managed to not only raise awareness of rare diseases but to secure the commitment of national

[2] Directive 2011/24/EU.

governments, supranational institutions, healthcare professionals and the private sector to tackle rare diseases.

So what did our representatives learn during the last membership meeting? What is on the horizon for rare diseases?

As noted above, there are still many unmet medical needs in the area of rare diseases that need to be addressed. Medical innovation has come a long way and innovative therapies, such as gene therapy and gene editing, bring hope of a cure for many severe and debilitating conditions. But at what cost? The development of new therapies in an area with a very limited patient population comes with considerable challenges, as demonstrated by the number of medicines obtaining orphan designation compared to those actually reaching patients. This can be due to different reasons, but it seems that one of the main challenges in proving these medicines' efficacy and safety is the limitation of data gathering due to the small number of patients, on which these medicines can be tested. This also brings uncertainty on whether European governments will be willing to reimburse these therapies, seeing the limited data. Currently, this doesn't seem to be the case. For example, of the two gene therapies approved in Europe in the past five years, only three patients have been treated[3]. More recently, we saw the example of a medicinal product developed for cystic fibrosis, a rare genetic condition, which was reimbursed in Ireland but not in Belgium and the Netherlands, as these countries deemed the benefits of this product as not being worthy of reimbursement.

This is why one of the biggest challenges for EURORDIS, and for many other patient organisations like the EHC, will be to ensure that patients can access novel treatments. EURORDIS is hopeful that data gathering on rare diseases will be facilitated by the further implementation of the European Reference Networks. In fact, these networks not only have the potential to bring together health care professionals and researchers, but to also gather data to advance research, which could be fed into the development process of novel therapies. However, many questions remain, including how these networks will run, seeing the limited funds that have been allocated for them, as well as how they will handle the legal challenges of data sharing in view of the implementation of the new data protection regulation.

Another opportunity that EURORDIS sees as potentially facilitating access to novel and innovative therapies is increased cooperation in the area of economic assessment of novel medicines. However, this could also backfire, as seen in the example with the cystic fibrosis medicines cited above. Still, EURORDIS sees great benefits from increased cooperation amongst European health technology assessment (HTA) agencies, which would prompt early dialogue between medicinal product developers, HTA and regulatory agencies. This is to ensure that developed novel medicines focus not only on safety and quality, but also on economic viability. On this note, EURORDIS launched in February a reflection paper to consider novel strategies for research, market access and post-marketing surveillance for novel and innovative orphan medicinal products (see EHC May newsletter).

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[3] Hirschler, B. Drugmakers' hopes for gene therapy rise despite tiny sales in Europe. Reuters. 8 August 2017. Accessed online: https://reut.rs/2vfhz5S

Encouraging European collaboration has been one of the mantras of the work done by EURORDIS and this has certainly never been truer than in the last six months, when EURORDIS launched the Malta Declaration. In this document, EURORDIS calls upon Member States to increase cooperation to ensure access to specialised health care services and diagnosis, stimulate research of rare diseases and speed up access to treatment for patients. In particular, this could realistically be achieved through the European Reference Networks and this is why in the same document, EURORDIS also calls for support of this project from national governments. All of these initiatives are meant to plant the seed for a renewed European action on rare diseases, taking the efforts of the past twenty years to the next level. Again, EURORDIS is not only focusing on European-level initiatives but also on ensuring the buy-in from national governments.

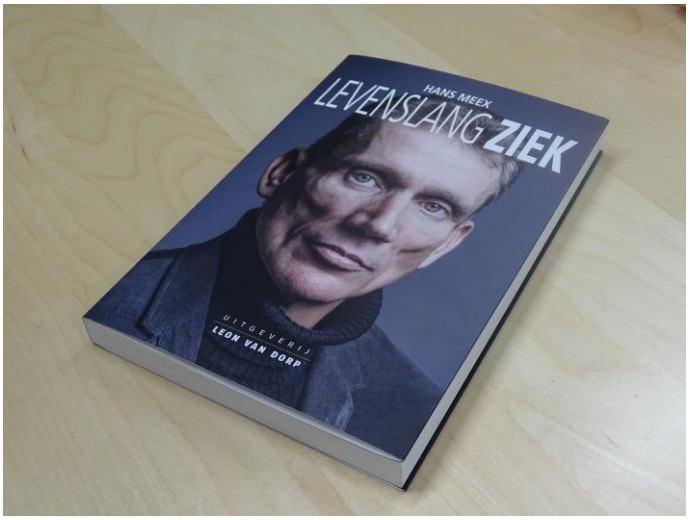
Finally, the EHC is delighted to see that continued attention will be given to the inclusion of patients' voices in all stages of medicines' lifecycles, from development to post-marketing surveillance. The work of EURORDIS has paved the way for many other patient organisations in this area. However, although patients' voices are more recognised than 20 years ago, patient representation is still lacking in many agencies (in particular at national level) and patient opinion is not always taken into consideration. This is most likely due to the perceived conflict of interest related to medicine funding, most often coming from the pharmaceutical sector. But that is a topic for a whole other article.

In short, many challenges that need to be addressed remain, from unmet medical needs to data gathering and economic evaluation of new medicines. It is also needed to remind ourselves that many patients with rare diseases, including in the bleeding disorder community, are still facing basic problems, such as getting a correct diagnosis and being able to consult with specialised health care professionals. There is still enough work for all of us.

Life journey: the good, the bad, the ugly and the beautiful

A look at the book 'Levenslang Ziek' (translation: Lifelong Sick), reviewed by Jo Eerens, EHC Membership Officer

You don't need to be an author to publish a novel; having a personal story can make for a plot that inspires and provokes thought. The remarkable autobiography of Hans Meex, a member of the EHC Dutch NMO, is that kind of a book – a wonder of survival and exploration of life in all its glory and misery.



The autobiography of Hans Meex, 'Levenslang Ziek'

Hans is a haemophilia patient, who was born in 1965. In those years, haemophilia treatment was still in its early stages of development and the main "prescription" was ice and rest. As most haemophiliacs that grew up in that time, Hans suffered from frequent bleeds that caused joint damage early on in his childhood and that left life-long consequences to endure. Later, the tragic events of the 80s did not spare him and he was infected with HIV and hepatitis C (HCV) through his haemophilia treatment. Both infections severely threatened his life but after a long battle with kidney and liver insufficiency, as well as heart failure, Hans recovered. It is in this time of "rebirth" that he wrote his story.

The autobiography is a positive testimony to what a person can push through. Hans is living proof of resilience, which in part is empowered by the support of his family and his strong character – he is a bit of a rebel, one might say. Throughout his youth and beyond, he would often stand up to the health system and doctors and nurses who, at the time, refused to listen to patients' concerns and dismissed them as insignificant.

Today, telling his life story has become a mission. And in that mission, I picked up on three main messages. One, you should never give up, even if you suffer from lifelong illnesses. It's true that you need the help and support of people who love you and who stand by you, people who make you feel free and make you believe in yourself. But in the end, you have to walk your own path and forge it towards where you want it to lead.

The second message is one of warning about what can happen when a medical professional simply becomes part of the system and treats the patient as inferior to him. Doctor-patient communication is of extreme importance and its absence can lead to horrible consequences (complications, further health damage or even death). Doctors should pay attention to what their patients are trying to tell them and establish an equal relationship, in which both work towards best approaches and outcomes.

This leads directly to Hans's third point on how this could best be accomplished through comprehensive care – care that includes the patient, doctors, nurses, psychologists and family members.

Currently, the book 'Levenslang Ziek' is only available in Dutch. Those who decide to read it, won't regret it – it is a book that you cannot put down!

Thank you, Hans, for this very intriguing and engaging testimony and to Cees Smit for sharing the book with me.

Upcoming Events

EHC events

September 15th – 17th EHC Tenders and Procurement Workshop – Open to NMOs only
Sofia, Bulgaria

October 5th European Inhibitor Network Workshop – Open to NMOs only
Vilnius, Lithuania

Directly before the EHC Conference, on October 5, a pre-conference workshop *"Finding ways for better inclusion of people with inhibitors in the life of the NMO"* will take place in Vilnius, Lithuania. The workshop will aim to explore the challenges with ensuring participation of people with inhibitors (PWI) in the life of the EHC NMOs and seek to **develop tailored action plans for inclusion** and involvement of PWI in the NMOs taking part in the workshop. As other EHC community-oriented events, this workshop will seek to be as **interactive** and **engaging** as possible, and aim to provide ideas, examples and tools for inclusion and involvement of PWI, offer peer support and stimulate cross-border initiatives.

A relatively small group is essential for the success of this workshop, therefore for this event, which we hope will be the first in a potential series, the EHC has invited selected NMOs. Furthermore, in order to achieve the best outcomes, two representatives from each of these NMOs will be represented – senior NMO leader and a person with inhibitors or a caregiver from the respective NMO community.

October 6th – 8th EHC Conference – Open to all
Vilnius, Lithuania

Message from Egidijus Šliaužys, President of the Lithuanian Haemophilia Society, EHC NMO:

We are honoured to host this event for the second time and look forward to welcoming all of you!

The first time the EHC Conference was held here was in 2009. Since then, the treatment of haemophilia in Lithuania has changed significantly – the availability of FVIII increased from 1.5 IU to 5.1 IU per capita.

We are excited that this autumn, when the European haemophilia community members gather for the EHC Conference, we will exchange experience and the latest information about the treatment of this disease. We hope that this important event will inspire and motivate all of us to continue to do good and meaningful work in the future.

To view the Conference programme, please visit http://www.ehcconference.org/2016-01-09-15-17-45/programme-5.html

November 16th – 19th EHC workshop on New Technologies in Haemophilia Care
Lisbon, Portugal

November 28th — EHC Round Table on Usage and Measurement of Extended half-life (EHL) Factor Concentrates and Novel non-replacement therapies
Brussels, Belgium

November 28th — Launch of the EHC PARTNERS programme
European Parliament, Brussels, Belgium

November 30th – December 03rd — EHC 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

February 7th – 9th, 2018 — 11th Annual Congress of the European Association of Haemophilia and Allied Disorders (EAHAD)
Madrid, Spain

May 20th – 24th, 2018 — World Federation of Hemophilia (WFH) World Congress 2018
Glasgow, Scotland

Announcements

The European Haemophilia Consortium (EHC) is delighted to announce that its 2017 Annual Survey will focus on women and bleeding disorders. The survey will focus on three areas: it will try to identify the number of women affected by bleeding disorders in Europe; it will try to identify what diagnosis, treatment and care is available to them; it will try to assess whether the needs of these women are properly understood and met by both clinicians and NMOs. Unlike previous years, this year survey will be composed of three questionnaires: one for women affected by bleeding disorders, one for EHC NMOs and finally one for clinicians caring for these women.

We are planning to launch the survey during the 2017 EHC Conference, where a copy of the questionnaire for NMOs will be available at the EHC Women's Working Group booth. Following the Annual Conference, the survey to women affected by bleeding disorders and clinicians will also be launched.

EHC Inhibitor Summit Registration:

As the registration for the EHC Inhibitor Summit was open mostly during the summertime, not all of you managed to recruit and register your participants on time. Therefore, we have decided to extend the registration deadline until the 20th of September!

We remind NMOs that you can register up to 4 participants for this event. Please https://www.ehc.eu/events/ehc-inhibitor-summit-2017/ for more details!

LOOKING FOR A SUPERHERO!

In the framework of the European Inhibitor Network (EIN) project, the EHC is seeking to inspire and encourage the youngest members of our inhibitor community, who often may feel discouraged and lonely. And who is better for this job than their own superhero?!

For this reason the EHC is happy to announce an autumn artwork competition for children to design the superhero of the EIN – INHIBITOR MAN/WOMAN! We see him/her as someone, who encourages children with inhibitors in the difficult situations they face, teaches them about their condition, gives positive example and joy for their daily life!

THEME

Inhibitor Superhero and his/her adventures (e.g. doing sports, flying, supporting children with inhibitors)

ELIGIBILITY

Children 7 – 15

REQUIREMENTS

Do:
- Submit only one drawing
- Use paper in the A4 size, horizontal or vertical
- Provide artistic interpretation of inhibitor superhero you have imagined
- Use any artistic technique (e.g. pencil, crayon, watercolor, collage, scratch-board etc.)
- Submit your original creation

Do not:
- Use photographs, tracings, computer-generated artwork
- Submit freehand copies of other people's work
- Put any lettering, signatures or initials on the front of the design

JURY

The 2018 EHC Inhibitor Superhero will be chosen by the EHC Inhibitor Working Group. Artwork is judged on quality and artistic interpretation.

MAILING ADDRESS

Please send your drawings to:
European Haemophilia Consortium
Rue de l'Industrie 10
B-1000 Brussels
Belgium

Please indicate *"Inhibitor Superhero Competition"* on the envelope!
Do not forget to put your name and return address on the back.

KEY DATES

Competition opens **1 September**, competition closes **1 November**.

WINNERS ANNOUNCEMENT

The winner will be announced on the last day of the EHC Inhibitor Summit 2017.

PRIZES

All the artworks will be exhibited during the EHC Inhibitor Summit 2017. The winning Inhibitor Superhero will inhabit EHC's on-line platform for people with inhibitors! All children who participate will receive a small reward.