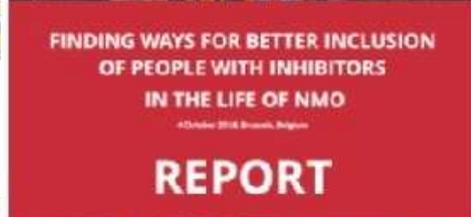




# **EHC NOW!**

## **Compilation Newsletter**

**September 2018 – January 2019**



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## A message from the President and CEO

*Written by Brian O'Mahony & Amanda Bok*



As you are probably aware, we now gather our weekly EHC Now! Updates and publish into one compilation three times a year. Each week we gather the current, most topical news to share with our members - event reports, National Member Organisation (NMO) updates, treatment updates and education, updates from members of our comprehensive care team across Europe who have shared their expertise with us and so much more. It seems each week becomes busier; each week we decide what information we need to prioritise to share with our members. This is an extremely luxurious position to be in and it is in no small part down to the fantastic, dedicated and ambitious work carried out by all members of our community - patients, volunteers, clinicians and other stakeholders. The final half of 2018 was no different, with many exciting events and developments from the EHC and our NMOs. The future continues to shine bright for people with bleeding disorders in Europe; through advocating, learning together and uniting our community in our goals, we can ensure that we achieve the most effective and efficacious treatment for people with bleeding disorders in Europe.

### Events

The last quarter of 2018 saw us deliver some of our most meaningful and well attended workshops and conferences; including our Tenders & Procurement Workshop where participants came together to learn the skills necessary to take an active part in their national procurement process. We also hosted our EHC Conference in Brussels, which we successfully co-hosted with our Azeri colleagues. The Conference was a huge success with a record attendance. It was also an opportunity to bring together different cohorts of our membership such as women with bleeding disorders – where we announced the first ever European Conference on Women and Rare Bleeding Disorders, which will take place from 24-26 May 2019 in Frankfurt, Germany. We strongly encourage all of our members to attend this worthwhile and informative event, which is one of a kind if Europe if not in the world! For further information please be sure to read page 8. Patients with inhibitors were also represented at our EHC Conference, specifically during the pre-conference workshop, which brought together patients and NMO leaders to ensure the inclusion of people with inhibitors in NMOs. Our final Round Table of the year had us looking forward to the future treatment landscape and how patients, NMOs, healthcare providers and other stakeholders will need to prepare for the changes that lay ahead in using these novel therapies. Towards the end of 2018, we held our third Inhibitor Summit in Barretstown, Ireland, again with record attendance, which placed the spotlight on the needs of people with inhibitors in our community. To begin 2019, we reunited participants from the five years of Youth Leadership Workshops to expand their training on advocacy and reignite the passion and commitment of this motivated and talented group. We are indebted to the volunteers, patients and NMOs who commit their time, expertise and resources to attend these meetings to further educate themselves and others – this continues to empower and strengthen our community, so thank you!

### EHC & NMO News

During our EHC Conference we said our heartfelt thanks to outgoing Mr Jordan Nedevski who has been succeeded as VP Finance by Ms Minette van der Ven from the Netherlands. We look forward to working with Minette while Jordan, who has been with us since 2007, will also remain a big part of our community, close friend and volunteer. We expanded our community and welcomed our 46<sup>th</sup> NMO with the addition of Tajikistan, we are sure you will join us in warmly welcoming our new NMO into the EHC community and we look forward to meeting members of their community. We also heard

about some exciting events and initiatives happening across our NMOs such as the Nordic Haemophilia Society's meeting, an international basketball camp by our friends in Lithuania and the newly announced youth group in Macedonia. It is a great encouragement to see our NMOs working together, but also developing programmes and initiatives locally to improve the quality of life for people with bleeding disorders in Europe and to ensure that all cohorts of our community are served. Challenges remain for some of our NMOs, for example the Haemophilia Society in the UK are currently working on their public inquiry into contaminated blood and facing an upcoming Brexit. We understand the challenges and difficult circumstances our friends in the UK - and others across Europe - embark on and always ready to provide support where we can!

As you can see there has been a lot of activity in the last few months and we hope that this EHC Now! compilation will help you to feel informed and part of the ever-growing, developing and changing patient community we are so very fortunate to serve. We would also like to extend our thanks to our corporate partners without whom our many activities would not be possible, and invite you to read their individual messages on to this, our last, 2018 EHC NOW! newsletter compilation.

With our best wishes for a healthy and successful 2019!

## EHC News

### EHC welcomes Minette van der Ven as its new Vice-President Finance

*By Laura Savini, EHC Public Policy Officer*

*During the EHC 2018 General Assembly Minette van der Ven, EHC was elected as EHC's new Vice-President (VP) Finance.*

Many of you probably already know Minette as she has been active not only in the Dutch haemophilia patient organisation (Nederlandse Vereniging van Hemofilie-Patiënten - NVHP), but also in many EHC events. Minette is in her mid-thirties and has been a member of the, EHC's Dutch National Member Organisation (NMO) since being a teenager. From 2010 until 2018 she sat on NVHP's Board and right now she is an active volunteer. Minette also suffers from Glanzmann thrombasthenia, a rare platelet disorder.



*The Dutch cycling squad arriving in Stavanger*

One of the activities that Minette has been very involved with in the past few years, and that many of you may be familiar with, is the Bloedverwanten



*Bloodrelatives, Minette is first from the left*

(Bloodrelatives) initiative. In 2016, a group of ten Dutch volunteers affected by rare bleeding disorders, including Minette, set off to cycle from the Netherlands all the way to Stavanger, where the EHC conference was taking place. Two weeks and 1000 kilometres later, the group arrived for the beginning of the Conference.

This initiative had two objectives, well three, actually: one was to promote and raise awareness about the practice of safe physical activities for people with rare bleeding disorders; the second was to fundraise for the NVHP to help patients in countries with lower treatment standards; and the third was simply to take part in a great adventure and create friendships and long-lasting memories. In fact, it took the team a year to train and prepare both physically and mentally for their endeavour. Throughout this whole period the group recorded their progress and shared it via social media. They also got local media (TV and radio) coverage to further raise awareness, not only about the initiative but also about bleeding disorders in general.



*Minette (right hand side) and Evelyn Grimberg arriving in Brussels ahead of the 2018 EHC Conference*

However, the adventure didn't stop in Stavanger as Minette and Evelyn Grimberg (another volunteer who took part in the Bloodrelatives initiative) cycled *again* - this time from the Netherlands to Brussels ahead of this year's EHC Conference to continue to promote the safe practice of sport for people affected by rare bleeding disorders. The Bloodrelatives also lives on as the funds raised by the initiative were used to support 'blood-relatives' in Georgia!

Minette has a background in finance and is a chartered accountant. As such, her motivation for running for the position of VP Finance was to put her professional skills and expertise to work to ensure the long-standing financial health of the EHC. Ultimately though, Minette put her candidature forward because by volunteering with the EHC, she hopes that she can help men and women in the community across Europe to have a better quality of life.

*The EHC is delighted to welcome Minette as its new VP Finance, and asks everyone to join us in giving her a warm welcome. You can meet Minette in person at various EHC events throughout 2019. We also extend our heart-felt thanks to outgoing VP Finance, Jordan Nedevski, who first joined the EHC SC in 2007 and served as VP Finance for eight years!*



*The EHC newly elected Steering Committee, Minette is fourth from the left. Former chair of the EHC Medical Advisory Group, Paul Giangrande, is centre right, next to Brian O'Mahony.*

## 2018 EHC Workshop on Tenders and Procurement

*Written by Laura Savini and Amanda Bok*

*From 7-9 September, some 20 volunteers from across Europe gathered in Amsterdam, the Netherlands, to attend the third and final edition of the EHC Workshop on Tenders and Procurement, an event made possible thanks to an educational grant from Pfizer. The objective of the weekend was to equip patient representatives from EHC National Member Organisation (NMOs) with the tools, skills and reflexes to take part in their national procurement process of medicinal products for the treatment of haemophilia and other rare bleeding disorders.*

### **Smooth sailing?**

We realise that spending two days listening to health economic concepts is not the most appealing way to spend a weekend, especially when in Amsterdam! Yet participants were actively engaged and involved because they know the landscape is changing.

On the one hand, the economic crisis from 2008 has taken its toll on European health care systems and has left payers increasingly wary of novel and expensive medicinal products. On the other hand, haemophilia has never seen so many varied and innovative treatment options coming its way. This makes for a perfect storm in which payers are critical of the real added value of novel therapies and an unwillingness to reimburse limits patient access. One of the workshop participants, himself from a national health care system, made this point strongly over the weekend. It was eye opening for participants to see that through his eyes haemophilia is an expensive-to-treat condition for which effective therapies already exist.

*"If you don't get involved in your national procurement processes, there may as well not be novel products because they'll just pass you by,"* warned EHC President Brian O'Mahony over the weekend. Motivating words for a workshop that builds on five previous years of EHC training in the area of economics, tenders and procurement. That is how important it is to our community to get patient representatives actively participating in the room when national decisions are made about the procurement, organisation and delivery of treatment and care. It is an entirely achievable goal, said Brian over the weekend, arguing *"it is easier to teach a person with haemophilia about health economics than it is to teach a procurement specialist about haemophilia."*

Following this impetus, much of the tender workshop was spent presenting participants with novel ways to demonstrate how new technologies in haemophilia care can help to increase trough levels while achieving savings for health care systems in the long-run. This is not straightforward and, at first sight, novel technologies can be perceived by the untrained eye of a general procurement specialist as expensive and un-necessary. Therefore it is crucial that patient representative be heard and be in the room when decisions are made with regard to the procurement of novel therapies.

### **Get up stand up!**

Bob Marley could not have rolled up his sleeves better himself. After going through a '101' on how to get involved in the tender process, participants reviewed both existing and novel therapies with a focus amongst others on health economics, safety and efficacy, tendering processes, and finally: practice! practice! practice! In fact, during the workshop we spent the entire Sunday morning carrying out a variety of practical exercises such as mock tenders. These looked at comparing standard treatment with extended half-life treatment and with non-replacement therapies. They certainly gave everyone in the room lots of food for thought (!) - and were by far the preferred part of the workshop, according to their feedback!

### ***The EHC Tenders and Procurement Workshop is here to stay***

You read it correctly! The EHC is not done training its members on the topic of economics, tenders and procurement. As made evident above, we believe that this is of crucial importance to the community and therefore we will continue to give it much weight and attention in our and our members' advocacy work! This is why the EHC is planning a new three-year series on tenders and procurement focusing in even more depth on novel therapies. The series started in September 2019 so stay tuned to the various EHC social media channels for updates and more information!



## **EHC announces first European Conference on Women and Rare Bleeding Disorders**

The EHC is proud to announce it will hold its first Conference on Women and Rare Bleeding Disorders. The event will be held in Frankfurt, Germany, on 24-26 May 2019.



To mark the opening of registrations, the EHC released a video promoting the upcoming conference. This event is open to all and further information can be found [online here](#).

**Early bird registration fees end on 22 February!**

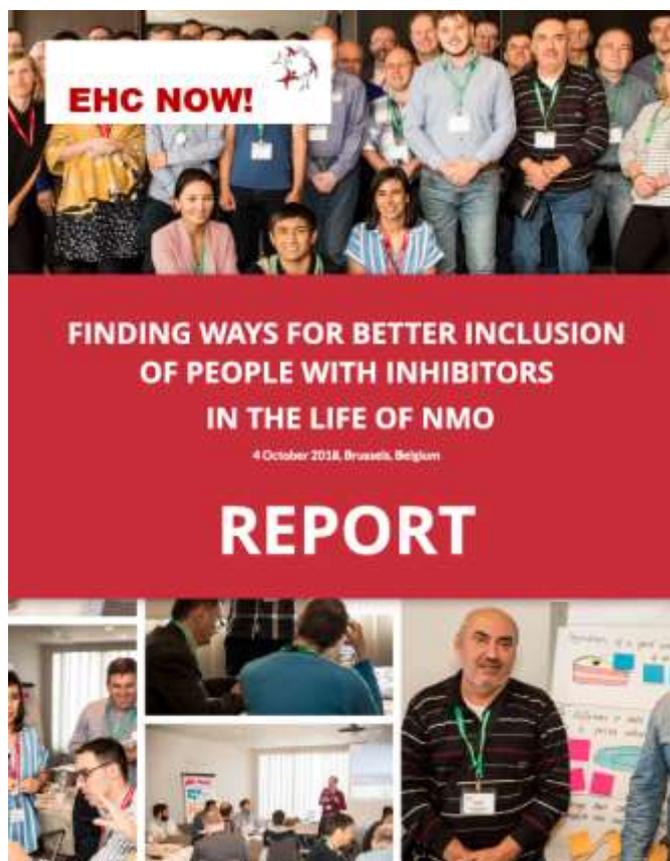
## EHC Pre-Conference Workshop: “Better Inclusion of people with inhibitors in the life of the NMO”

*Written by Kristine Jansone, EHC Programmes Officer*

The EHC Pre-Conference Workshop “**Finding ways for better inclusion of People With Inhibitors in the life of NMO**” took place on 4 October 2018, prior to the EHC Conference in Brussels. It brought together more than 30 NMO representatives, both people with inhibitors and NMO leadership, as well as the EHC Inhibitor Working Group members and observers.

The workshop aimed to explore the challenges with ensuring participation of people with inhibitors (PWI) in the life of the EHC National Member Organisations and seek to **develop tailored action plans for inclusion** and involvement of PWI in their NMOs. Through a variety of interactive methods, the programme of the workshop focused on the diverse aspects of addressing the needs of people with inhibitors in the EHC NMOs.

[Read the full report online here.](#)



### Video highlights of the EHC 2018 Conference

In November the EHC released a video with the highlights of the EHC 2018 Conference. The video can be viewed [online here](#).

## Looking forward to the EHC Conference

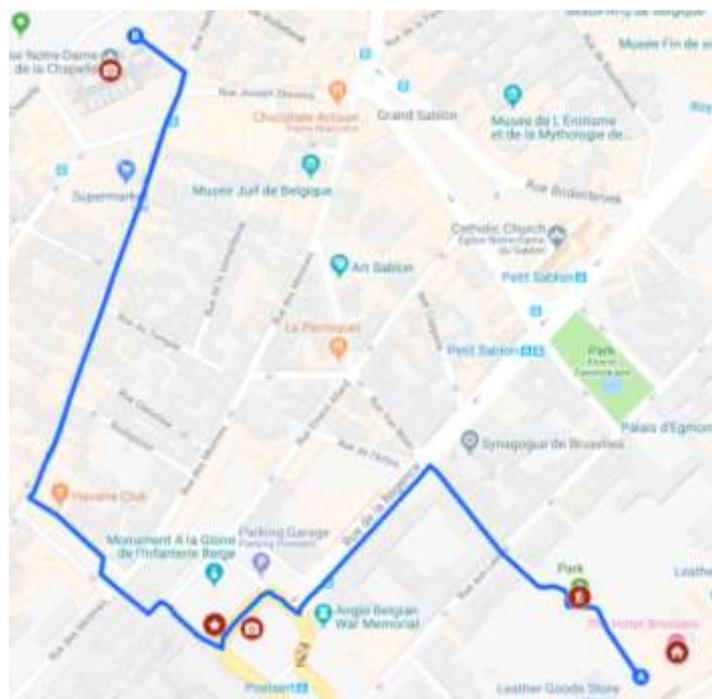
*Interviews taken by Laura Savini, EHC Public Policy Officer*

Ahead of the European Haemophilia Consortium (EHC) Conference (#EHC2018) EHC staff members give their perspective on what to expect from the event and how to make the most of the visit to Brussels.

### Saskia Pfeyffer, EHC Office Assistant

*What are you most looking forward to in the next EHC Conference?*

For this year's edition, I am particularly excited about **the EHC stand**. In fact, we have come a long way in terms of stands and with each event it is getting better and better! This year the EHC stand will be divided into three sections: one for EHC activities, one for the Inhibitor Working Group (IWG) and one for the Women and Bleeding Disorders (WBD) Committee. Although the IWG and WBD Committee are two of our 'newest' groups (established in 2017 and 2018 respectively) they are also two of our most active groups and have already been developing a wealth of activities. I really invite everyone to come by and talk to these groups. In particular for representatives of our National Member



*Map of Brussels showing the Marolles area nearby the conference venue*

Organisations (NMOs) it would be interesting for them to come by and see how they can get involved or bring back some of their activities to their countries. As for the part of the stand focusing on EHC activities, I do not want to spoil much but we are already thinking of our 30<sup>th</sup> anniversary celebration, which will take place next year and we have developed something special, so come by and help us in preparing the festivities!

*What would you advise our delegates to do in Brussels during their free time?*

I really like **the Marolles area**. It is an area located walking distance from the Conference venue, filled with flea markets, antique shops, vintage clothing shops and lots of small cafés and restaurants. It's really great to take a stroll around on a Sunday.



**Kristine Jansone, EHC Community Programme Officer**

*What are you most looking forward to in the next EHC Conference?*

On Thursday, the day before the start of the Conference, we will be holding our second **pre-conference inhibitor workshop**, during which we will be discussing how NMOs can be more inclusive of people with inhibitors in their national activities. This workshop has doubled in attendance since last year and I am really looking forward to energising discussions and fresh ideas!

*What would you advise our delegates to do in Brussels during their free time?*

To complement Saskia's suggestion above (to visit the Marolles), I would say that it's really worth going there via **Place Poelarts** where the imposing building of the Justice Palace is located. From there you'll have a great viewpoint of Brussels. This is particularly nice at sunset, so if you're looking for a place to walk in between sessions and dinner, I would totally recommend to go and check it out. From there you can take an elevator to the lower part of the city, the neighbourhood of the Marolles.

**Declan Noone, EHC PARTNERS Consultant**



*What are you most looking forward to in the next EHC Conference?*

For me the **Saturday night dinner and dance** is the best part of the Conference. I love watching the switch from serious dedicated work by clinicians, patients and NMOs taking the time to enjoy each other's company. It is these connections and interactions that have built the strength and unity in this community over the last 30 years.

*What would you advise our delegates to do in Brussels during their free time?*

I do not live in Brussels and every time I visit I am in and out of the city in a day, but if our delegates want to experience something typical, I would recommend to **taste one of the many beers that Belgium has to offer**. In particular, I would recommend to try a Kwak beer in its specific beer glass. Just make sure to not wear your best clothes because it can get messy!



**Fiona Brennan, EHC Community Programme Officer**



*What are you most looking forward to in the next EHC Conference?*

Personally I have been very much involved in the preparation of the **Youth Debate Symposium** taking place on Friday at 16.30 hrs. I love to see how our youth volunteers grow from attending their first EHC event, which often is the Youth Leadership Workshop, to this moment. It is quite intimidating to debate against some of the most prominent clinicians and patient leaders in the haemophilia community but, so far, all of them have stood up to the challenge and I am sure that this year's participants will do an equally fantastic job!

*What would you advise our delegates to do in Brussels during their free time?*

I am also not based in Brussels but from my many visits, I found out that Brussels is in fact a very green city with many parks. I would highly recommend to take a stroll in one of Brussels' many park and in

particular in **Parc d’Egmond**, which is adjacent to the Conference venue. It’s a small park featuring a lovely café where our delegates will be able to stretch their legs and – hopefully – enjoy some sunshine!



#### Jo Eerens, EHC Membership Officer

*What are you most looking forward to in the next EHC Conference?*

As membership officer, I am really looking forward to **meeting with all of our members**, in particular those that do not have the opportunity to attend other EHC events. The Conference is always a great time to catch up and learn what our members are busy with in their own countries.

*What would you advise our delegates to do in Brussels during their free time?*

If you are visiting the Marolles, I would recommend to stop by **Kapellekerk (église de la Chapelle)** where Bruegel the elder (a famous Flemish painter) is buried. This year marks Bruegel’s 500th anniversary and to celebrate, many exhibitions and events are taking place around Belgium and in Europe. Currently there is a small exhibition of Bruegel in the church itself where characters from his paintings have been placed all around the church.



*Statue of Bruegel the elder outside Kapellekerk*

#### Laura Savini, EHC Public Policy Officer

*What are you most looking forward to in the next EHC Conference?*



I think this year’s **session on gene therapy** will be very exciting (Sunday at 12.00 hrs). This is, in particular, as many of the gene therapy trials are entering phase II or III and will start recruiting patients in Europe. I believe it is extremely important that our members are aware and informed of this revolutionary medical technology and I am delighted that we will have a patient describing his experience going through the treatment.

*What would you advise our delegates to do in Brussels during their free time?*

Although you may think this is a bit cheesy, I personally think that no visit to Brussels is complete without some **culinary experience!** Brussels has an impressive selection of restaurants and if you’ve never had it, I think you should try the classic dish of mussels with chips and beer! Many restaurants in the downtown area will feature this dish, I would say that Chez Léon in the city centre is a landmark for this particular dish.

#### Amanda Bok, EHC CEO

*What are you most looking forward to in the next EHC Conference?*

Everything! Absolutely everything! I’m also looking forward to events just starting already - we’ve been preparing for so long and it’s the busiest Conference we’ve ever organised! But since you insist, I might give some special mentions. In addition to the usual programme and everything the others have mentioned, we also have our **#thisway**





Brussels city map featuring some of Amanda's recommendations

**awareness-raising bike ride** tomorrow, in which half the team and myself will participate - I can't wait! It builds on the fantastic Dutch Bloodrelatives initiative from 2016 and includes some of the original cyclists! I'm so impressed in particular with Minette van der Ven and Evelyn Grimberg who will start cycling in The Netherlands, meet us in Leuven, and then join our Leuven-Brussels #thisway bike ride to the Conference. The other thing happening this weekend, behind the scenes, is that we have two separate film crews on site who will be filming, respectively, an **educational patient-focused series on gene therapy and a documentary about women and bleeding disorders**. But really, I'm looking forward to everything and everyone. It's a great community - and a great team - and I'm looking forward to coming all together again this weekend.

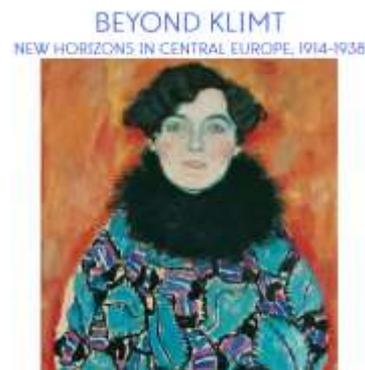
*What would you advise our delegates to do in Brussels during their free time?*

I'm one of those rare people that is really in love with this city. I recommend two things. For those who want to stay near the hotel, I highly recommend strolling behind the hotel between **Petit and Grand Sablon** all the way to the **Royal Palace**. In between you'll find lots of cafés, restaurants, art galleries and museums! There is a new exhibit that I really want to go to called **'Beyond Klimt' at the Bozar**, includes works by Josef Capek, Egon Schiele, Oskar Kokoschka, László Moholy-Nagy and 75 other artists. I'm a big fan of Kokoschka myself! And as our Conference dinner will take place at the Bozar, people will already know how to find it! For others who would rather leave the touristic area and go 'slightly' further, I recommend **Place St Boniface** (still within walking distance!) where my favourite Brussels bar/brasserie, [Ultime Atome](#), serves a great selection of Belgian beers!

*Thanks to all EHC staff for their recommendations. We are excited to meet and welcome all delegates in a few days!*

For more information about the EHC Conference visit <http://conference.ehc.eu>

For recommendations on things to do in Brussels go to <https://visit.brussels/en>



## EHC 2018 Conference highlights and looking towards the year ahead

*Interviews taken by Laura Savini, EHC Public Policy Officer*

*Following the EHC 2018 Conference, we asked some of our Steering Committee members to tell us what were their highlights of the conference and what they were most looking forward to in the coming year.*

### Naja Skouw-Rasmussen



*Some of the Programme committee members for the EHC Conference on Women and Rare Bleeding Disorders*

For me the highlight of the conference and of the work ahead is **the increased visibility and focus on women with rare bleeding disorders**. During the conference we had the women's booth and a full session focusing on women. In the margins of the conference we met with the EHC Committee on Women and Bleeding Disorders as well as the organising committee for the **EHC Conference for Women and Bleeding Disorders taking place next year on 24-26 May 2019**. The work of the committee and the conference are two things that I am

looking forward to as well as the development of a **movie on Women with Bleeding Disorders in Europe, which will be available in 2019**. Keep an eye on EHC social media for more information on all of these!

### Minette van der Ven

I was thrilled to be **elected VP Finance** and become even more involved in this community. I also echo Naja's sentiment with regard to the increased work on Women with Bleeding Disorders. I am looking forward to familiarising myself with all the practical aspects of the day-to-day management of the EHC and I hope I will be able to **make meaningful contributions to ensure the running of the consortium is even smoother**. Finally, I am really looking forward to supporting the whole community and to **bringing my experience and dedication wherever they are needed!**



*The new EHC Steering Committee (Minette van der Ven is fourth from the left)*

### Michael van der Linde

For me the highlight of the weekend was the **cycling to the conference** and the **youth debate**. I really believe that it

is important to continue to highlight the benefits of physical therapy and an active lifestyle, which are beneficial for everyone and in particular for those with bleeding disorders. We need to continue to spread the message that there is a way to practice sport safely, even when access to treatment is not

optimal. This can be done together with both haematologists and physiotherapists who play a key role in comprehensive haemophilia care. This is why I was delighted that Pfizer donated its symposia to the EHC so that we could partner with the European Association for Haemophilia and Allied Disorders (EAHAD) to really stress the **benefits of physiotherapy**. I believe this was a successful symposium with an important message.



*The #thisway campaign cyclists arriving in Brussels*

With this conference I also conclude my role on the EHC Youth Committee and I am very proud of the work this committee has achieved and each year I am amazed by the talent, energy and motivation of youth volunteers from across Europe. There is no better place to demonstrate this than the Youth Debate, a symposium donated by Sobi to the EHC. This year the performances on the debate were stellar and this truly shows that training youth is worth all the resources, time and energy that the EHC has been

investing in it in the past few years.

This coming year I will continue to look ahead at the EHC youth-related activities, which will now include the **Youth Fellowship Programme**, the **Youth Leadership Workshop** and **the work of the new Youth Committee**. I am excited to see the youth activities grow, this is a real investment into the future of the European bleeding disorders community.

### **Brian O'Mahony**

For me **the record attendance at the EHC Conference** (with over 375 delegates!) is a true statement of the good work that the EHC volunteers and the staff are doing throughout the year. I was pleased to see the sessions being very well attended, in particular on **novel therapies**. There is a real sense of excitement in the community for what is coming and this will be discussed in depth in the **Workshop on New Technologies in Haemophilia Care** that the EHC will hold later in November. We also received very positive feedback on our new **newsletter on novel haemophilia therapies**. The committee managing this publication has met in the margins of the conference and they will publish two short updates at the end of this year and in February 2019 before publishing a fully revised long-version of the newsletter in July 2019 after the ISTH Congress.

For the year ahead, I am also looking forward to continuing the work to **support the inhibitor community both through the European Inhibitor Network and the Inhibitor Summit**. This often-overlooked part of the community needs all the support it can get, in particular at a time when exciting novel therapies for inhibitors are coming to European markets; we need to ensure that our inhibitor



Brian O'Mahony as Inhibitox,  
the inhibitor superhero

community will be able to access these novel therapies.

Finally, the year ahead will be very important our **PARTNERS programme (Procurement of Affordable Replacement Therapies – Network of European Relevant Stakeholders)**. We are going to mark the one-year anniversary of the programme during our next **Round Table of Stakeholders on 'Switching from standard therapies, where do novel therapies fit in?'** taking place on 20 November in the European Parliament. Dr Cristian Busoi will be hosting the Round Table, which is fitting seeing he was one of the Members of the European Parliament (MEPs) that supported the launch of PARTNERS almost a year ago. We will also continue our advocacy work with governments in the PARTNERS programme to promote sustainable purchase of adequate levels of haemophilia therapies as well as the organisation of comprehensive haemophilia care. For this, we are delighted to be partnering with our colleagues at EAHAD. Finally during the Conference we announced that Roche officially joined PARTNERS, we are really excited about this and we are hoping that this will contribute to better access to treatment for people with inhibitors in PARTNERS countries.

#### **Olivia Romero-Lux**

I want to echo a few things that were already mentioned, such as the work for women with rare bleeding disorders and the importance of physical activity. In this regard, **I am hoping that we will be able in the future to build-in ahead of each conference some kind of activity to highlight the importance of the practice of sport and physical activity.**

I also very much enjoyed the talk by Alison Dougall on **oral health** and I am hoping that the EHC could increase its activities in this area as I think that there are still many misconceptions and taboos surrounding oral health in people with bleeding disorders.

Finally, meeting the community is always such a pleasure and really energising for continuing the work of the Steering Committee. It is an opportunity to 'take the temperature' on what our NMOs are doing and what topics are at the heart of our members' concerns. I think that the EHC should also definitely look at what it can do for people with **Von Willebrand Disease**.

However, nothing is set yet, so stay tuned for more updates.

*Radoslaw Kaczmarek, Miguel Crato, Thomas Sannié and Alain Weill (ex-officio) were not available for quotes.*

# Seven things we learned from the EHC Round Table on 'Switching from standard therapies: where do novel therapies fit in?'

*By Laura Savini, EHC Public Policy Officer*

*On Tuesday 22 November 2018, the European Haemophilia Consortium (EHC) held its third and last Round Table of Stakeholders of the year at the European Parliament in Brussels. The event was kindly hosted by Dr Cristian-Silviu Buşoi (EPP/Romania) and attended by Dr Miroslav Mikolášik (EPP/Slovakia), both long-standing supporters of the European bleeding disorders community. Dr Dan Hart, member of the EHC Medical and Scientific Advisory Group (MASAG) and Honorary Consultant Haematologist at Barts and the London School of Medicine and Dentistry chaired and moderated the event.*

*The topic of the event was 'Switching from standard therapies: where do novel therapies fit in?' Speakers tried to tackle the issue of exactly how clinicians and patients should decide when to switch from as standard half-life (SHL) coagulation factors to a series of novel therapies.*

## **1. It all starts with education**

Across all presentations speakers noted that clinician and patient education is key to ensuring that the correct decision is taken in relation to treatment regimens. Education should not be limited to the treatment options available but should be extended to having a clear understanding of how these medicines work and how they impact patients' health. Patients should be encouraged to learn about concepts of pharmacokinetics such as peaks and troughs as well as 'time under the curve.' In fact, clinicians believe that when patients understand how the treatment works and impacts them, they become more motivated to adhere to their treatment regimens and this will ensure better outcomes on their quality of life.

In addition, patients should be their very own advocates and be able to explain their treatment regimens and conditions to non-specialist clinicians in, for example, cases of emergency or when consulting non-haematologist health care professionals.

## **2. Personalisation and intensification are (or should be) the new normal**

Novel therapies offer unique opportunities for the personalisation of treatment. This should not mean the 'rationing' of treatment but rather its intensification to achieve higher protection based on the individual patient's metabolism, lifestyle and other characteristics. This may mean maintaining the same infusion schedule on extended half-life coagulation factors (EHLs) but using it to achieve higher trough levels thereby having better protection against bleeds. This is particularly interesting for patients with more severe bleeding phenotypes, those that wish to engage in more active lifestyles or those with bad joint damage.

## **3. Non-linear progression of treatment innovation**

The progression of haemophilia treatments from blood components to replacement therapies and beyond has moved exponentially. Viewing this progression as linear, however, implies superiority between the existing and novel therapies, which may be a misleading analysis. Instead, speakers

proposed that we now have a menu of treatment possibilities, options and choices for the real personalisation and individuation of treatment.

Of course, the reality is that in many countries novel therapies are not yet available, and in some they are available but only for a limited number of patients. Speakers stressed that SHL therapies remain a very safe and effective treatment and should still be considered even when novel therapies are available. Participants were also reminded that when launched, most EHL therapies had a similar price as SHL products, enabling some clinicians in some contexts to switch in order to achieve higher protection while using the same treatment budget. However, should SHL considerably reduce in cost, this would allow other reflections and choices to open up for clinicians and patients, for example enabling countries with limited health care budgets to provide increased access to treatment for their haemophilia patients.

The arrival of bispecific antibodies has been particularly innovative for haemophilia A patients with inhibitors who were previously not able to benefit from prophylaxis due to the short half-life of bypassing agents. However, it is important to note that bypassing agents will still be needed especially to treat acute bleeds and during surgery.

It is clear that we are entering a paradigm shift in the way we, as patients and clinicians, approach and consider treatment strategies and choices over their lifetimes. Increased personalisation will be the way of the future and will ensure that patients get the best outcomes from different treatment products.



*Participants during the Round Table*

#### **4. Moderate haemophilia patients need more attention**

Speakers noted that at the moment people affected by moderate haemophilia (i.e. patients with coagulation factor levels between 1 and 5 per cent) are the patients with the worst health outcomes. This is because in most countries these patients are not as prioritised as their severe counterparts and consequently are not provided with prophylaxis or home treatment and often face negative impacts on their joints. It is ironic therefore that, with the intensification of treatment, patients with severe haemophilia will effectively become either moderate or mild patients in the future. Therefore, isn't it time that patients with moderate (and even mild) haemophilia are also offered the same treatment opportunities? This is certainly a topic for discussion that, we foresee, will be recurrent in the coming years.

## 5. Patients need to be involved in the decision-making process

With all this innovation, the importance is increasing for patients to be actively consulted and involved not only in relation to their treatment regimens but also in the broader decision-making process on the organisation of haemophilia care and, in particular, the purchase of haemophilia treatments. With such different modes of action, novel therapies will be increasingly difficult to compare to each other and to standard therapies. This will result in challenges with assessing benefits and values. Most certainly new methods of analysis will be needed, said speakers.

## 6. Monitoring is needed

At the end of the day, these are novel therapies for which we still do not have much real world evidence with regard to safety and efficacy. Only real world clinical practice will give us more information on when these products are best used and their real safety profile. Therefore speakers recalled the importance of regularly monitoring patients on novel treatment, not only for inhibitors, and sharing this information in the treatment community.

## 7. Continuing European advocacy

In the concluding remarks, Dr Hart noted that as these treatments are not yet available everywhere and, as some countries still do not meet the minimum standard of haemophilia care, it is important to continue strong advocacy work in Europe.

In this context, the EHC's PARTNERS programme (Procurement of Affordable Replacement Therapies - Network of European Relevant Stakeholders) remains of high importance as it seeks to increase access to treatment in countries where European consensus standards are not yet met. The PARTNERS programme was launched one year ago in the European Parliament and will release its one year progress report and reflective White Paper shortly.



*Dr Buşoi, centre, kindly hosted the EHC Round Table*

## European inhibitor patients gather in Barretstown for the 3<sup>rd</sup> EHC Inhibitor Summit

*By Laura Savini, EHC Public Policy Officer*

*From 6 to 9 December close to 150 people gathered in Barretstown, located an hour outside of Dublin in the beautiful Irish countryside. Their meeting: the European Inhibitor Summit, a yearly event organised by the European Haemophilia Consortium (EHC) for people affected by inhibitors in rare bleeding disorders. The Summit was also the last EHC event of the year and the culmination point of European Inhibitor Network (EIN) activities led by the EHC Inhibitor Working Group (IWG).*

### **What's the EIN, anyway?**

In 2015, the EHC started the [EIN](#) to support an often under-served and marginalised part of the European bleeding disorders community: people with rare bleeding disorders and inhibitors.

Haemophilia and other rare bleeding disorders are in and of themselves rare diseases, which means that they only affect a very small number of people in each country. Even rarer are those affected by inhibitors. These are patients that often would not have met anyone else like them in their lifetime just because of the rarity of their condition and this singularity will contribute to the feeling of isolation and disempowerment. Furthermore, until recently people with inhibitors were faced with very limited and costly treatment options, which meant that unlike their non-inhibitors counterparts, their health, and in particular their joint health, would be severely damaged leaving them disabled. This in turn had a negative impact on their own and their family's mental and general health as well as social and economic life. The idea of the EIN was to build a network to provide a peer support system where people affected by inhibitors can meet, exchange and seek support from each other, as well as empower and train them to become strong and active advocates in their own right.



Chief amongst the EHC's current objectives for the EIN is to identify an Inhibitor Ambassador in each country that could be a liaison or contact person for all other people affected by inhibitors in that country, and that would be able to provide support and information on inhibitors and in turn liaise with other patients in Europe.

### **The Times They Are a-Changin'**

Besides building a community, the objective of the EIN is also to provide information to patients about novel treatments. Regular readers of this newsletter will know that [this is a very exciting time for haemophilia because so many novel and innovative treatments are being developed](#) and are starting to appear in European countries. Some of these treatments will also be effective in people with inhibitors and can be used prophylactically. This will give our patients with inhibitors a real alternative and provide much-needed options for their treatment and quality of life.

However, care in rare bleeding disorders is not only limited to treatment and also includes other comprehensive care services such as physiotherapy and dental care. This is why the EHC runs a [monthly exchange with healthcare professionals](#) specialised in the care of people with inhibitors where participants can ask them questions from the comfort of their homes. The video conference is open to all those with an interest in inhibitors and runs every last Thursday of the month.

Another early EIN objective was to provide consensus recommendations and guidance on managing inhibitors in order to establish common standards across Europe. To achieve this, the EHC partnered with clinicians to develop the [European Principles on Inhibitor Management](#), which were published earlier this year. The EHC is currently working on the production of multiple advocacy tools, including posters and an animated video, based on these Principles with which to reach various stakeholder groups across Europe and promote their implementation.

Finally, the EIN wished to raise awareness of the reality of life with an inhibitor. As we all know that *'an image speaks a thousand words,'* the EHC produced 20 minutes of moving images through its documentary ['Inhibitor Stories'](#) filmed in 2017 and launched last year. It is a short and compelling movie that captures patient, parent and healthcare provider stories from across Europe.

### **Third time's a charm**

So: back to our Summit. It is meant to be the culminating point for the European inhibitor community. It is an opportunity to meet in person with peers who are facing similar situations but also to get information from experts on the management of inhibitors, as well as the latest medical and scientific updates in that management.

For the third year running, the event was held in [Barretstown](#), a not-for-profit camp designed for children who are suffering from severe illnesses and disabilities, so that everything is designed to accommodate people with reduced mobility and special needs. Barretstown was funded by actor Paul Newman and is part of the SerioüsFun Children's Network of camps across the world.

The EHC was very proud to see that this year numbers of participants (compared to the first year) almost doubled! The atmosphere was one of a family reunion! We were thrilled to see so many familiar faces of returning participants and to witness their personal progress on their inhibitor journey. We were equally delighted to see many new faces and how they were immediately welcomed and taken under the wings of the 'returning attendees.' For our part, the initial objective of the Summit, to create a community and peer-support system, seems to be going in the right direction. The event was a mix of lectures on scientific, medical and care topics (such as for example stress management). The programme also featured several peer-to-peer sessions, enabling participants to have their own private space to freely discuss and exchange on issues of concern or day-to-day challenges. Children and teens had parallel programs around the camp that include a mix of adapted sports, arts and crafts activities as well as lectures on how to manage their inhibitors, for example via good dental hygiene that is adapted to people with inhibitors.

The camp is attended by lots of families, therefore during the day, there is free time built into the programme for families to spend some quality time together and do some activities that they wouldn't normally get to do, such as rock climbing, zip lining or horse-back riding under supervision and guaranteed safety. At the end of each day, participants are welcomed into smaller home groups, designed to allow participants to digest the day's learnings and events, and address any questions, concerns or other matters in a smaller and more intimate setting.

All of this is made possible thanks to a wonderful team of volunteers and staff from Barretstown but also thanks to the volunteers of the Irish Haemophilia Society (IHS) who have massively contributed in bringing participants to the camp, entertaining the children and helped to ensure that everything went as smoothly as possible.

We also wish to give our heartfelt thanks to all of the speakers and moderators that attended the meeting, giving generously of their free time over the entire weekend. We were really delighted to welcome amongst them representatives from the Canadian Haemophilia Society and also the US National Haemophilia Foundation. Since the beginning of the EIN, it was really important for the EHC

to learn from, and exchange with, other Societies on inhibitor programmes, and we are delighted that our colleagues from across the pond have been so enthusiastic about taking part in our Summit and welcoming the EHC to theirs. This ensures that our collective work on inhibitors is coherent and we can all learn from each other and share best practices.

A special thanks also goes out to our corporate partners Roche, Sanofi-Genzyme and founding partner Shire for their financial support of the entire European Inhibitor Network.

Finally, our biggest thanks go to all of the participants, who make this event truly special! We leave you with some quotes from this year's Summit and hope that this will encourage many more to attend next year!

**What our participants said about this year's summit:**

*"It is exceptional achievement, just wish we had heard of you years ago. Feel as though we have struggled alone for such a long time."*

*"Thank you for an amazing couple days! Attending the summit has been life-changing. There are so many uniquely different "experiences" of living with an inhibitor, but also so many similarities. It is really quite powerful to see how families in other countries cope with life with an inhibitor. Will take what I have learnt here and implement into our programming in the US inhibitor programme."*

*"Excellent summit and helping people mix together was much better. I love that all the summits have been fantastic and EHC take on the feedback and suggestions each year to improve when I already think it was good. Well done!"*

*"I am meeting the most wonderful people every year and it is amazing :)  
Thank you!"*

**References**

- [The EHC European Inhibitor Network](#)
- [Ask the expert](#)
- [EHC Newsletter on novel treatments in haemophilia](#)
- [EHC Inhibitor Stories](#) (a short movie about people affected by inhibitors in Europe)
- [The European principles of inhibitor management](#)
- Pictures from the event will be published in the coming days on the [EHC Facebook page](#)
- Want more? [www.ehc.eu](http://www.ehc.eu)
- For more information about the EHC Inhibitor Programme you can get in touch with Kristine Jansone (Kristine.jansone[at]ehc.eu), EHC Programme officer

## EHC Youth Leadership Workshop Alumni Event

*By Charles Kinney, EHC Communications Officer*

*For this week's EHC Now! we report on the first edition of the Youth Leadership Workshop Alumni event that took place in Amsterdam over the weekend.*

Amsterdam provided the backdrop for the EHC Youth Leadership Workshop Alumni Event held 25-27 January, 2019. With over 30+ participants from all over the EHC network, the EHC Youth Leadership Alumni Workshop Event proved to be a successful continuation and celebration of five years of youth training and advocacy. The event was aimed to build, assist and encourage participation and engagement in NMOs for the youth and to focus on expanding the participants' knowledge and skills in terms of advocacy for patients with haemophilia and related blood disorders around Europe. The theme of the event was **WWWWHW (who, what, when, where, how and why)**. The intensive three-day event, always high energy with the EHC Youth Leadership Alumni, was not only a "hands-on" workshop for the EHC advocate membership to gain more knowledge about being an effective advocate, but an opportunity to network and continue to build a community for young people with bleeding disorders in Europe.

The event focused on practical and pragmatic exercises building on the five-year history of EHC Youth Leadership Workshop.

Under the leadership of EHC's Fiona Brennan, the event opened with Valentin Brabete from Romania leading a session about lifelong learning developed by the Alternative University in Bucharest. This was followed by an informative and highly useful session by EHC CEO Amanda Bok and the UK Haemophilia Society's Board of Trustees Chair, Clive Smith, using an excerpt from Martin Luther King Jr.'s *I Have a Dream* speech, combining the power of advocacy with the power of speech. Clive highlighted that credibility is the byword, 'whatever it is they need to know, make sure it is the truth – you want to be accurate, reliable, understandable and actionable.' The session also emphasised that by using and understanding rhetorical devices your audiences and participants can advance their message.

Amanda demonstrated that by providing proof (facts) through data (here's the literature!) and a paper trail, you can gain equal-footing stating 'data is a universal language, everyone can use it, everyone can understand it.' Amanda also led a very popular communications style exercise to use for finding the best spokesperson, which focused our attention on how to communicate effectively with people with different and sometimes opposing communication styles to achieve your goal. Communication is advocacy and advocacy is an art. Clive Smith and EHC Steering Committee member Thomas Sannié, spoke about real-world experiences in NMOs and critically examined where we can improve but also how the training can be applied to refine and analyse our advocacy skills.

Women have also stepped up and are taking a bigger role in advocacy than ever. Olivia Romero-Lux spoke about NMO updates. Naja Skouw-Rasmussen from the Danish Haemophilia Society stressed that, "Women can and should take a bigger role in advocacy. Women need to be informed and be concrete in their solutions in women's bleeding disorders. Stronger advocacy and participation in your NMO is the way forward."

Some of the main take-aways participants were offered to advance their advocacy skills were the importance of preparedness, providing proof by collecting data and using the literature, offering viable solutions and the importance of sustaining long-term relationships both internally and externally. Last

but perhaps most importantly, all external advocacy requires internal advocacy. The NMO was referred to as a living organism; we need to be flexible and adaptable and continue to grow together.

Reactions from participants were highly positive. In addition to the next steps in advocacy, participants gained practical, real-life experience from roleplaying situations experienced by advocates. These included both internal and external advocacy issues, constructive discussions inside NMOs, engaged discussions with Ministries of Health and an intensive session of what to do with the press. Robbert-Jan T'Hoën from the NVHP said of the roleplaying experience, "When you sit you see, but when you do you learn."

Facilitator Stefan Radovanovic from the Serbian Haemophilia Society said, "It's good to see how far we've come. When we started, this was relatively new for us. We understand what advocacy is and we want to go further."

It's not an understatement to say that the Youth Leadership Workshop Alumni are the future. When asked what he gained from the experience, Youth Leadership Alumni member Alex Teinburg, from the Estonian Haemophilia Society said, "Confidence. Practical experience, like body language and English-language clues from experienced advocates like Clive Smith, give us more tools we need for greater participation."

The next Youth Leadership Workshop will take place on April 5-7, 2019 in Amsterdam.

Watch a short video summarizing the event [online here](#)





## EHC Welcomes 46th National Member Organisation: Public organization “Haemophiles” of the Republic of Tajikistan

*By Azizi Najibi, Chairman of the Tajiki NMO*

*The public organisation “Haemophiles” of the Republic of Tajikistan was officially formed in 2017 and became a member of the European Haemophilia Consortium (EHC) during the 2018 General Assembly. Mr Azizi Najibi, Chairman of the organisation tells us more about how the association was created as well as challenges for people with bleeding disorders in Tajikistan.*

First of all let me express my gratitude to you for all your care about us and for your intention to introduce us to our brothers in blood from the European haemophilia community.

Before our patient organisation became an official legal entity in 2017, the NMO applied several times since 2015 for registration and submitted its draft statutes to the Ministry of Justice. The final edition of the final edition of the statutes on 10<sup>th</sup> November, 2016, which was approved by the Ministry of Justice of Republic of Tajikistan as of 21<sup>st</sup> February 2017.

The primary aim of any public organization is to protect the rights of and advocating for the interests of the community which created this organisation. To that end, the leadership of our organisation stands up to defend the rights and interests of its members to ensure equal opportunities with all other citizens, to solve the problems of disabled people, to organize charitable activities, to improve treatment and prophylaxis of the disease and to provide social rehabilitation for the patients.

The main founder of our public organisation is the General Assembly. According to our statutes, the General Assembly elects an executive director for a term of five years. I, Azizi Najibi Sharifbek, was elected as the first chairman of the organisation.

In spite of the fact that factor concentrate is not used in the treatment of haemophilia in Tajikistan, the overall situation is tolerable, as the government constantly cares about hemophilia patients, and they receive plasma or cryoprecipitate. We believe that switching to factor concentrates is only a matter of time. At this point we have been liaising with the Ministry of Health and Social Care of Republic of Tajikistan to implement treatment with factor concentrates in the city of Dushanbe. This area includes 76 patients with haemophilia A. This initiative is supported by World Federation of

Hemophilia (WFH), which provided the first tier of humanitarian aid to help to achieve this goal. The second tier was scheduled for the second part of 2018.

In Tajikistan, there are 457 patients that are officially recognized. Amongst them 204 are under 15 years old or younger. 417 patients are diagnosed with Haemophilia A while 31 are diagnosed with Haemophilia D, and eight with Haemophilia AB.

All adult patients with haemophilia are the members of our organisation; also, the organization maintains permanent contacts with parents and caregivers of children with haemophilia. We invite parents and caregivers to take part in all of our activities. The leaders of the central office and regional branches have haemophilia themselves.

The Republic has three regions: Kuhistani Badakhshan (Gorno-Badakhshan) Autonomous Region, Sughd and Khatlon Region. In addition to Dushanbe, there are a number of cities and tens of districts in Hisor valley and Rasht valley. To date, we have representatives in Sughd and Khatlon regions. Our main goal is the protection of rights and interests of people who suffers from such congenital



disease as haemophilia, and after weighing up the pros and cons, we decided that we cannot do without support of International community [of haemophilia].

The way toward the international communities was opened for us at the Almaty Forum, held on 26-28<sup>th</sup> May 2017 in the city of Almaty, the capital of the Republic of Kazakhstan. At that Forum we were introduced to Mr Yulian Tsolov, representative of WFH, who delivered our appeal to Mr Alain Weill, President of the WFH.

In October, 2017, a working group from WFH arrived to Dushanbe to review of our work. During the visit, we organized a conference and workshop for people with hemophilia and healthcare professionals, under the title of 'When time afflicts a limb with pain ...'<sup>1</sup> During this conference, attended by more than 400 participants, we approved an appeal to the President of Republic of Tajikistan 'On the [need of] adoption of the Law on Haemophilia.' This conference was highlighted by local mass-media. We wish to stress that the key role in our achievements at international level was played by the Russian Hemophilia Society, led by its president, Mr Yury Zhulev. We are exceptionally grateful to Mr Zhulev for his support towards our community.

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<sup>1</sup> The verses of Saadi Shirazi, whose poem is placed at the entrance of United Nations headquarters: 'When time afflicts a limb with pain, The other limbs at rest cannot remain' – *remark by the translator*

On 25<sup>th</sup> May 2018 during the WFH Congress in Glasgow, Mr Alain Weill handed me over the certificate stating that our organisation had become a member of the WFH.

At Bishkek Forum in September, 2018, we met with Mr Tsolov who helped us to pass our appeal, addressed to Mr Brian O'Mahony, to the representative of European Haemophilia Consortium.

Our organisation is planning to learn from your experience in advocating and protecting rights and interests of patients with haemophilia. We have intention to pass the requirements of the patients to the government and legislative bodies. The organisation strives, whenever possible, to offer legal support to the patients, and we already achieved some good results in this regard. In 2019 we are planning to organise a 'School for patients.' Although, we have no experience in holding such a schools, we'll use the experience and skills of our Russian colleagues. We are bound to be successful, as we are not alone, and all our brothers in blood, from the entire planet, stand with us.

As the say goes, to make a great hit, you need to gather your fingers together, and we already proved this proverbial wisdom to be true, because, when we created our organization, we no longer need to fight for our rights alone. During this period we already achieved the results each of us could only have dreamt of when acting separately.

The organisation has its own lawyer, and we stand behind those patients who were unable to receive their [disability] pension, and after re-assessment by [medical expert] commission, more than 20 people began to receive this pension again. Also, the organization prepared an official appeal to the Tajiki General Prosecutor's Office regarding the inappropriate actions undertaken by the prosecutor officer in the Asht District of Sughd Region toward the patients with haemophilia.

After registering the Haemophilia Society of Republic of Tajikistan, haemophilia became one of key topics in national mass-media such as newspapers, radio and TV who constantly highlighted this topic. Despite some achievements, we also experience certain constrains and difficulties. First of all, the members of our executive board work as volunteers, due to the lack of founding. To date, our operating account remains empty. Furthermore, we lack basic office devices such as computers, printers, fax machines coupled with lack of access to the internet and phone connection. All of this negatively impacts the efficiency of our work. To date, people with haemophilia in the Republic do not have access to such comprehensive care specialists such as orthopedists, dentists, surgeons, etc. Patients in Tajikistan are also in need of nurses and laboratory scientists, who should master their skills at specialised trainings in European clinics.

In conclusion we wish to say that, in spite of all the obstacles, we look into the future with optimism, as we believe in the development of medicine.

Azizi najibi Sharifbek is the Chairperson of the Tajiki NMO. He originates from Sagirdasht, a village in the Darvaz District, in the Kuhistani Badakhshan Autonomous Region of Tajikistan. As a higher education he graduated from Tajik National Conservatoire with a degree in Folk Songs. He has two children, a daughter Aziza and son Umar.

*The EHC wishes to warmly thank Azizi for this introduction. On behalf of all EHC members, we wish to welcome to the Tajiki NMO to the EHC.*

## 2018 Meeting of the Nordic Haemophilia Societies

*Interview taken by Laura Savini, EHC Public Policy Officer*

*Each year the haemophilia societies from the Nordic countries (Denmark, Finland, Iceland, Norway and Sweden) meet to touch base on their respective activities, discuss common issues and exchange on best practices. For the 2018 edition, countries' representatives met in Helsinki, Finland. Naja Skouw-Rasmussen, member of the Steering Committee of the European Haemophilia Consortium (EHC) attended this year's event and reports on the main discussions and take-aways from the event.*

*Naja tells me that there are many similarities within Scandinavian countries not only culturally and from a socio-economic point of view, but also with regard to how haemophilia care is organised and delivered. The standard of haemophilia care is also famously high in the region. This is not only due to the generally excellent health care services but also to the contributions made by key haemophilia clinicians who work together through the Nordic council, a clinician-driven body that develops guidelines and principles of care for the region.*

### **Much ado about novel treatments**

*So what were the 'hot' topics discussed by these patient communities over the weekend? Seeing the standard of care is very good, one of the most discussed topics over the weekend was the availability of novel therapies. Participants focused in particular on how government procurement bodies will look at these treatments and how they might prioritise availability given some unanswered questions. Another issue that came out of the weekend is that, because the standard of care is so good, many patients do not invest in learning about haemophilia and how the treatment works. Therefore, these patient organisations feel that there was a real need for increased patient education.*

### **Compliance, compliance, compliance**

*In this regard, one of the most exciting sessions looked at treatment compliance in younger patients. Excellent standard of care means that some patients have never experienced a single bleed. Naja told me that for some patients this creates a real disconnect as they are 'sick' but have never experienced any symptoms and therefore this can create a false feeling of being safe from the disorder. This sense of safety can push younger patients to 'test' their limits and see for how long they can go without treatment, hence creating the number one issue in haemophilia care in developed countries: lack of compliance to prophylactic treatment.*

*One suggested strategy to improve compliance is to ensure that children as young as seven or eight are put in charge of their own treatment and are taught to self-infuse. In fact, it is believed that this may help develop a routine and sense of responsibility that will carry on later in life. If self-care begins at around 15 or 16 years, it will coincide with adolescence and the appetite to break rules and push boundaries. The main issue with this strategy is, understandably, that many parents want to ensure that their children comply with their prophylactic regimen and so take responsibility for their treatment. The trick would be for parents to let go and give responsibility to their children.*

*With regard to compliance, an important role can also be played by patient organisations to strengthen activities that would include young children and their families and to create a peer support system to which patients can turn in case of need. Having strong ties within the community could be particularly valuable during teenage years when patients struggle more to manage their disorder.*

### **PK as a tool to increase compliance**

*On the topic of treatment management there were also discussions on understanding pharmacokinetics (PK). Again this was found of interest as many patients have little knowledge about*

their disorders and concepts like PK can help to improve compliance by underlying how treatment is 'processed' by their body and what levels of protection they can expect. The Nordic countries want to achieve a reality of 'no bleeds' but it should go hand-in-hand with an understanding of what haemophilia is and how the disorder needs to be cared for. In fact, at the moment many patients feel anxiety towards bleeds because they fear that, should they have a bleed, they would not recognise it or have any idea about how to treat it.

Naja also stressed that despite many patients receiving excellent health care and being in very good health, there are of course still patients facing hurdles and for whom life is not all that perfect; this is for instance the case for people affected by inhibitors.

All in all the weekend was spent highlighting the need for increased patient education, in particular with the youth, and exploring collaboration amongst countries to move forward together.

*The EHC would like to thank Naja Skouw-Rasmussen for sharing her impressions on the weekend and giving us a little insight into this year's Nordic meeting.*



*Participants at the 2018 Nordic Meeting*

## UK public inquiry into the blood contamination

*By Laura Savini with text provided courtesy of the Haemophilia Society UK*

*September was a month high in emotions for the haemophilia community in the United Kingdom (UK) as a series of preliminary hearings were held for the Statutory Inquiry into the Contaminated Blood Scandal. The events were opened by a commemorative ceremony in which survivors or relatives of those who passed away following the contamination were able to provide first-hand accounts of the personal impact the contamination had on their lives and on the lives of their loved ones. The hearings continued with opening statements about priorities for the Inquiry and procedural matters.*

The victims of this tragedy have been waiting for 40 years for an investigation that could provide them with answers as to how such a tragedy could have occurred, the lack of adequate response from authorities to these events and whether there was misconduct from government officials. The contaminated blood scandal did not only affect people from the haemophilia community but also people affected by thalassaemia and people who received blood transfusions.

The Statutory Inquiry into the Contaminated Blood Scandal was set up following an announcement by the UK Prime Minister, Mrs Theresa May, in 2017. In a statement in the UK Parliament she described the scandal as *“an appalling tragedy which should simply never have happened”*.

The UK Government later confirmed that the inquiry would have full powers, including the power to compel the production of documents and to summon witnesses to give evidence under oath. In February 2018 it was announced that High Court Judge Sir Brian Langstaff would be the full time Chair of the Inquiry. Speaking on his appointment Sir Brian said: *“Providing infected blood and plasma products to patients truly deserves to be called a major scandal. I intend, through this Inquiry, to be able to provide both some well-needed answers to the victims and their families, and recommend steps to ensure that it’s like will never happen again.”*

The Inquiry officially got underway on 2 July 2018, following the announcement in the UK House of Commons of the detailed Terms of Reference. Haemophilia Society UK Chief Executive, Liz Carroll, said: *“The Community has campaigned long and hard for this Statutory Public Inquiry to be held. With this announcement of the Terms of Reference, the way is now finally clear for the Inquiry to begin and justice to be served [...] The Society is committed to seeking the truth and will not rest until justice is achieved for all.”*

Sir Brian Langstaff said: *“What is difficult to comprehend is the sheer scale of what happened. The numbers of people, both adults and children, from all walks of life who were infected by hepatitis viruses, or HIV, from clotting factor or transfused blood runs into thousands. At least as many more – including partners, children, parents, families, friends or carers – have been affected [...] This may have happened principally in the 1970s and 1980s, but the consequences persist today with people continuing to feel the mental, physical, social, work-related and financial effects [...] Many of the people infected and their families have battled for years to understand what happened and how they have been treated since. I aim to put the people who have been infected and affected at the heart of this Inquiry. I am determined to get to the truth and where necessary will use the Inquiry’s power to compel witnesses to explain their actions.”*

Barry Flynn, Chair of the Trustees of the Haemophilia Society UK said: *“As Chair of the Haemophilia Society, and a member of many years standing, I welcome the official start of the long-awaited Statutory Public Inquiry into the contaminated blood scandal. As part of this community I am acutely aware of the impact this has had on individuals, their families and loved ones. We have waited decades for this*

*and want the truth to be revealed and for people to find justice and, hopefully, closure." He then added: "Through my role as Chair and the conversations I have had with those who live with, or have lost relatives through, this tragedy I know there are many who do not feel they can be heard as they are not natural 'campaigners.' Our role at the Haemophilia Society is to help the 'quiet voices' of those affected by bleeding disorders in the UK and, through our international networks, ensure best practice is shared across the world." Barry concluded by saying: "I am very pleased that, at last, the Inquiry is getting underway and that, whatever the truth is, it comes out and we all get the answers we have deserved for so long."*

The Haemophilia Society UK has also developed a [website](#) to provide further information into the inquiry itself and all of the advances can be followed on its official [website](#), [Twitter](#) and [YouTube](#) account where the hearings will be live-streamed.

The story has got much attention within the UK and internationally and notably the [BBC](#) has been covering in depth the progress of the inquiry and the impact that this tragedy has had on the lives of those affected.

*The EHC wishes to thank the Haemophilia Society UK for the information provided here above and will strive to provide its members with regular updates on the Inquiry. For more information about this please contact [publicinquiry@haemophilia.org.uk](mailto:publicinquiry@haemophilia.org.uk) directly or get in touch with representatives from the UK National Member Organisation (NMO) at <https://haemophilia.org.uk/>*

## International basketball camp for people with haemophilia 2018

*By Egidijus Sliauzys, President, EHC Lithuanian NMO*

*As you know the European Haemophilia Consortium (EHC) has been trying to promote physical activity through its #thisway campaign, which has mainly featured cycling as an activity to stay fit. Now, there are of course many other ways to safely practice sport and stay fit, even when affected by a rare bleeding disorder. This is why we are particularly excited to feature this week's EHC Now! story on the Lithuanian basketball camp. This activity has now become a bit of a tradition going on for several years and including not only young people from Lithuania but an increasing number of international participants coming from neighbouring countries and from across Europe. We hope you will enjoy this article!*

For the third year already, the EHC Lithuanian National Member Organisation (NMO), LŽSHA, held an international sports and fitness camp during which many people with haemophilia had the opportunity to enjoy freedom of movement, exercise and wellbeing while learning new basketball techniques. The camp was held from July 16-22 in Birštonas at the "Tulpė" sanatorium haemophilia rehabilitation centre, a place specialised in the treatment of haemophilia.

This year we were delighted to welcome to our camp people with haemophilia from Lithuania, Poland, Ukraine, Belarus, France, Slovenia and Bulgaria. The camp, as always, was a great opportunity to prove to the world and to ourselves that contrary to common misconceptions, people with haemophilia can also partake in physical activities. In fact, anyone who is getting the right treatment may sometimes even be able to do more than a healthy person. This was proved once again by our LŽSHA team, which this year also included international guests. For me, it's always great to watch everyone playing together with their blood brothers!

Through this camp, we really demonstrate that our common 'problem,' haemophilia, really unites us



*Participants at the basketball camp*

and that united we can achieve a lot! Professional trainers and clinicians helped our participants to strengthen their physical condition. In fact, some of the participants of this year's camp came from countries with very limited access to treatment and were, therefore, in pretty bad physical condition. But even in this short time they managed to improve their physical condition through a series of physical trainings specifically designed for them and including exercises in the gym and swimming pool, training on the

basketball court and morning exercises on the grass.

A week is a fairly short period of time to learn the basics of basketball, but everyone has worked hard to prove that they can really dribble, shoot and score. I'm glad that this year a lot of young people from our NMO participated in the camp. Seeing them working hard in the gym and basketball court really gives us hope that the association's basketball team has a beautiful future ahead and that it will soon be filled with many new members.

Naturally, during the camp we held friendly matches between the Lithuanian and the visiting team and I am pleased to report that the LŽSHA team defeated our guests 64 to 42. Once again, we proved we are a basketball nation and that the lack of coagulation factor does not matter when you have basketball flowing in your veins since birth! Guests from abroad were thankful to participate in the camp and enjoyed meeting new people, doing some exercise and sharing experiences. During this camp we also started to film a documentary to showcase to the public and to people affected by haemophilia in Lithuania that sport and haemophilia go hand in hand when adequate treatment is available.



*A participant ready to shoot*

After all of this everyone felt considerably better and, in fact, we are proud to report that nobody got injured during this week and that there were no bleeds into joints or muscles and for this, I would like to thank our doctors and trainers who took such good care of our participants! These include: the sanatorium "Tulpė" team comprising clinicians, physiotherapists and nurse Onutė Malinauskienė, who helped participants with factor infusions. Our thanks also go to haematologist Rasa Kariniauskienė, who supervised our patients throughout the camp. Many thanks to physiotherapists Greta Baronaitė and Viktorija Jušinskaite for their exercises, kinesio taping and massages. Finally, a very big thanks to basketball coach Justas Ališauskas, who worked with the guys on the basketball court.

Last but not least, I would like to leave you with some comments from our international guests about the camp:

*Gaétan from France said: "I used to play basketball when I was a child and in my teenage years but due to an ankle injury I stopped playing basketball ten years ago. For me being able to play basketball again with people affected by haemophilia felt incredibly lucky. It was a true honour to be invited to the camp, to meet new people from all over Europe and to better understand the importance and level of physical activity in different countries. The workouts during the basketball camp were quite intense and they really helped me to understand how loaded exercises and heavy practice could be performed without injuries. This really showed that anyone can play basketball and I wish to sincerely thank LŽSHA and its members for their kindness and the time invested in this activity. I came back home having made new friends and with a strong willingness to promote physical activity for people with bleeding disorders."*

*Bartek from Poland said: "It was my third time attending this basketball camp and, as always, it was an amazing experience. The LŽSHA basketball camp gives us the opportunity to feel like 'normal people.' The everyday practices were not that easy but they showed us that we can really improve our basketball skills, stability and muscle strength through tailored exercises. The camp was attended by people from all over the Europe and sometimes even further. The beautiful thing about this event is that we all like each other and we feel some kind of unity. We can discuss our health problems with friends from other countries and find new ways to deal with them. We can also discuss how the health care system is working in our countries. The camp ends with two friendly matches between the two*



## Newly-formed youth group in Macedonia

*By Marija Aloa Vera Drzmanoska, member Hemolog, the EHC Macedonian National Member Organisation (NMO)*

*The Macedonian NMO introduces its newly-formed youth group.*

I am delighted to have the opportunity to be a part of our new Youth Group in Macedonia. We created it with the purpose of including younger members and allowing them to build deeper connections with each other, the NMO and society. This will encourage more civil engagement by the youth along with team work and compassion.

Since last month's creation, we have five members including myself and we are looking forward to including more soon. The members in alphabetical order are: Antonio Steva, Bujamin Dauti, Mirsad Jahiu, Vani Nuhi. All youth is welcome regardless of class, religion or social background.

The Youth Group proudly spreads its main message that youth engagement is essential to move forward as a community. Youth these days have different needs compared to the older generations.



*Marija, one of the members of the Macedonian Youth Group*

This means that their needs must be answered to allow them room for personal growth through increased involvement with the society and the NMO. As of now, all of our young members eagerly await tasks that will help make an impact and look forward to cooperating with the European Haemophilia Consortium (EHC) and the World Federation of Hemophilia (WFH).

Now, let me tell you a little bit about myself. My name is Marija Aloa Vera Drzmanoska and I come from a family that has a long history of haemophilia. I, myself, am a carrier of haemophilia A severe. After hearing about this disorder from a very young age, I am finally old enough to take measures within the community to help my family and other members suffering in the society.

I am especially proud to say that I am part of my own Civic Engagement Project from the United States Agency for International Development (USAID). I was given the opportunity to choose an area of interest that I wish to improve in the Macedonian society. The project includes educational and training programs funded by USAID and I cannot wait to share all the knowledge and experiences I gain with my fellow members in the Youth Group. I personally found raising awareness for bleeding disorders and motivating young people to volunteer to be of great importance and this is why I decided to make this the theme of my project. I am very thankful to be involved in such a project. Needless to say, such a project requires a lot of time, dedication and hard work. However, I am prepared to make such sacrifices that will help me evolve in the long run as an individual part of our society. It gives me the opportunity to lead people in the creation of a more inclusive and humanitarian community. With that being said, I look forward to seeing where this project leads in the next nine months and even more so about the results it will bring.

The Youth Group is still in its early stages but we think its creation is a huge step forward for Hemolog, the Bleeding Disorder Society of Macedonia. We see the phrase *"the whole is greater than the sum of its parts"* very applicable in our case. Instead of having all our young members contributing individually,

we believe that by working together they can reach new heights both for themselves and the community.

*The EHC works hard to invest in the patient advocacy and leadership skills of the future. This is why we have built a comprehensive youth leadership strategy that takes young leaders on a journey from the EHC Youth Leadership Workshops, to the EHC Leadership Conference and culminates in the EHC Conference, where we invite young patient leaders to debate with established community leaders. These workshops and trainings offer our young leaders the opportunity to explore the patient advocacy environment, to understand the patient-stakeholder roles and responsibilities, but also to develop relationships and share experiences with other NMOs in Europe. Our newest and exciting addition to our Youth Strategy is our Youth Fellowship Programme, which aims to get our trainees working together and to keep them motivated to be an active part of the community! We will establish project teams with participants from across Europe to achieve a mutually beneficial goal, while also enabling our young leaders to develop real hands-on experience and contribute in the right way to the European community.*

[Find out more about the EHC Youth Strategy on the EHC website.](#)

## What is haemophilia?

*Interview with Lino Hostettler, President of the Swiss Haemophilia Society; Interview by Raia Mihaylova, EHC Communications Officer.*

Having haemophilia is often met with misconception of what it exactly is. On the other hand, it can be a task to repeatedly explain or be difficult to know how to best approach the topic in certain environments, such as school or work. As a picture is worth a 1000 words, the Swiss Haemophilia Society created an animation video that illustrates what haemophilia is in simple terms with the aim to easily inform both children and adults. Lino Hostettler, President of the Society, explains how they came up with the concept and the process behind it.



**Raia Mihaylova (RM):** *Why did you decide to do such a video, what is its aim?*

Lino Hostettler (LH): The Board of the Swiss Haemophilia Society decided to produce a new informative video to share with our community. We brainstormed on several topics and then realised that there is no such video about the simple question "what is haemophilia?" We thought it was a good idea to create a simple and short video that explains haemophilia to everyone – may it be to show in school for the children, to show a friend, or anyone else.

**RM:** *How did you come up with the concept to do it as an animation?*

**LH:** We had the same producer work on this video as on the first one we did, which was on "what does half-life mean?" He came up with the idea to do it as a comic so as to make it suitable for both young and old. We think that this worked out pretty well!

**RM:** *What is the process of creating such a video?*

**LH:** We first came up with the idea and then met the producer. Together, we decided on the length of the video and if we wanted to use the same concept as before (animation). After that we prepared a script (the story in text form), which is the basis for the video. We tried to use as simple words as possible and to keep everything short. And of course, there should be a flow in the story. Then the producer started to create a storyboard with an idea. We took part in the whole process, letting him know if there was an idea we disliked and needed to be changed. Then the producer designed the final clip. Finally, we needed to decide on the voice over – if it should be a man or a woman, etc. Before the launch, we showed the video to friends to see what their reactions and comments were. It was a very interesting process!

*You can access the video in the below languages using the following links:*

- [English](#)
- [French](#)
- [German](#)
- [Italian](#)

## FEATURE ARTICLES

### Novel technologies in haemophilia care: EHC tried and tested methods to keep-up-to date with an evolving medical landscape

*By Laura Savini, EHC Public Policy Officer*

*The New Year is traditionally a time for taking stock of the year that is past and looking ahead at a year full of promise and exciting opportunities. This is also applicable to the field of haemophilia care in which we are seeing, like never before, many new treatments being developed and in late clinical trial phases. Some of these treatments will most likely come to the European region in the next few years, so this is the time to really familiarise yourself with all these new technologies and closely follow what is going on in this field. Of course doing so can be sometimes time-consuming, daunting and often one does not know where to begin to look for information. So, look no further, in this edition of EHC Now! we give you our tried and tested tips on how to familiarise yourself with novel technologies and stay-up-to date with developments in haemophilia treatment.*

#### **First up, get yourself a copy of the EHC New Products Newsletter**

The 'Novel treatments in haemophilia and other bleeding disorders: A period EHC review' is a newsletter issued by the EHC in which we provide you with extensive information on ALL novel technologies in development for the treatment of haemophilia but also other rare bleeding disorders. The first issue, published in May 2018, is [available on our website](#) and provides great detail on the modes of actions of these new technologies as well as information about the regulatory process to develop a new medicine.

The second edition of this review newsletter covering updates from May to December 2018 is currently being finalised and will be released shortly. It will not be as extensive as the first issue and will primarily give an update on advances in existing trials, medicines that have been licensed, clinical trials that have been started and ended. This newsletter also offers some points for reflection including, for example, the impact of gene therapy on patients in terms of how this will affect someone not only physically but also, for example, psychologically.

*The next issue of the EHC New Products Newsletter will be out in the coming weeks.*

#### **Check out Medscape**

In 2018 Medscape developed and launched together with several patient organisations including the European Haemophilia Consortium (EHC) a medical education programme on gene therapy in haemophilia. The programme is designed to educate healthcare professionals involved in haemophilia care such as haematologists and paediatricians to get up to speed with the latest developments in gene therapy in haemophilia. The training is free and counts towards continued medical education (CMEs) credits. This programme was supported by an educational grant from BioMarin. The EHC partnered on it together with the World Federation of Hemophilia (WFH) and the US National Hemophilia Foundation (NHF).

While that programme focuses primarily on healthcare providers, we believe that these educational videos are also interesting for patients with more advanced knowledge and understanding of gene therapy, as well as good tools for patients to take back to their healthcare providers nationally - so please help spread the word and the learning!

Find out more about [Medscape Gene Therapy education here](#). You will need to create an account to view the content but it's easy so don't let that stop you!

### **Get on to social media such as Facebook and Twitter**

Perhaps you view social media as a black hole for free time and a means to procrastinate on important work, and you've made it your New Year's resolution to stop wasting time on your phone!

We couldn't agree more on the need to use social media in a smarter way and to try to minimise daily screen time. However, if consulted in moderation, social media can also offer timely and broad updates on medical advances in haemophilia care, so we recommend choosing whom to 'follow' wisely! And while we're dishing out unsolicited advice, we personally recommend to follow the [Facebook page of Haemophilia News](#). This page compiles haemophilia-related news from across the web and is updated almost daily. The page, which is managed by a patient, gives an overview of global news in haemophilia ranging from medical advances to changes within the pharmaceutical industry to curious facts about haemophilia and other rare bleeding disorders.

We also strongly advise anyone with an interest in haemophilia to start following [@ProfMakris](#) on Twitter. This is the Twitter handle of Prof Michael Makris, Professor of Haemostasis & Thrombosis at Sheffield University and a member of the EHC Medical and Advisory Group (MAG). The information provided in Prof Makris' Twitter feed does not solely relate to haemophilia (although the theme is predominant) but also looks at scientific and medical information related to thrombosis, general research and education.

### **Attend the EHC Conference.... and if you want to get down to the nitty gritty of novel technologies, join us for the EHC New Technologies Workshop**

Now we're really getting ahead of ourselves, but in case you don't already know, the EHC organises a scientific conference each year during the first full weekend of October during which we provide a general update on scientific and medical advances in the field of rare bleeding disorders. This event is open to all and the [next edition](#) will be held on 4-6 October 2019 in Skopje, Macedonia. The official programme will be released shortly but we can tell you that it will certainly feature sessions on gene therapy, non-replacement therapies and extended half-life as well on their real world clinical use and future potential impacts.

Then, once you'll have attended the EHC Conference, you'll have gotten a taste of all the novelties and, we would imagine, that you would really want to get to the bottom of all of those technologies. That's what our 23-25 November 2019 workshop on New Technologies will be for! [This workshop](#) is only accessible to EHC patient members and designated healthcare providers and national authorities. It is comprised of two intense days of scientific presentations on novel therapies in haemophilia and other bleeding disorders, and their clinical use. The workshop however, also gives an opportunity to participants to have candid conversations and ask questions to world-leading experts on anything that may excite or concern them with regard to these new technologies. So if you are a curious patient, healthcare provider or national decision-maker, don't miss it!

The next Workshop on Novel Technologies in Haemophilia Care will be held in Athens, Greece.

*We hope that this brief overview will help our readers to get started on learning more about advances in haemophilia therapies. For further information do not hesitate to get in touch with the EHC office.*

## **NEWS FROM OUR CORPORATE PARTNERS**

The EHC would like to acknowledge and thank the 2018 corporate partners of the EHC Round Table of Stakeholders programme, which promotes dialogue and exchange between all stakeholders. In this spirit, we are pleased to share their news with readers in this section

### **Platinum:**

- BioMarin
- Pfizer
- Roche
- Sobi

### **Gold:**

- Bayer
- CSL Behring
- Novo Nordisk
- Shire

### **Silver:**

- Biotest
- Sanofi Genzime
- Spark

Building on our *Heritage* to Pioneer a *New Era* of care



## Global commitment to patients through community initiatives

Pfizer believes in partnering with the haemophilia community at all levels to support initiatives that aim to improve access, standards of care and education for people with haemophilia and their families.



Michael  
Makris

EAHAD President,  
EHC Medical Advisory Group

“Physiotherapy is important for persons with haemophilia during their entire lifespan.”



Piet  
de Kleijn

EAHAD Physiotherapists  
Committee, EHC Advisory Board,  
EHC Medical and Scientific  
Advisory Group

“There is unfortunately still a gap between the importance of physiotherapy and the actual implementation in haemophilia care worldwide.”

### Raising awareness of the value of physiotherapy in haemophilia

The Pfizer-sponsored satellite symposium *“Importance, recent developments and actual implementation of physiotherapy for haemophilia in Europe”* at the European Haemophilia Consortium (EHC) 2018 Conference was developed in collaboration with EHC and the Physiotherapists Committee of the European Association for Haemophilia and Allied Disorders (EAHAD). The symposium was designed to help raise awareness around the value of physiotherapy in haemophilia.

Co-chaired by Michael Makris and Piet de Kleijn, the programme covered a breadth of topics, including EHC and EAHAD Initiatives to Improve access to physiotherapy across Europe, the role of physiotherapy within comprehensive care, and approaches to implementing quality physiotherapy care in practice.

During this engaging symposium, the multidisciplinary faculty gave their perspectives on how physiotherapy can serve to improve the everyday lives of patients with haemophilia. Michael Makris and Piet de Kleijn discussed their involvement in the European Haemophilia Network (EUHANET) and EAHAD Physiotherapists Committee, respectively, highlighting the positive impact these projects have on patient care.

Lia Ramishvili (K. Erstav National Centre of Experimental and Clinical Surgery, Georgia) described how difficult, but also how important, it is to implement physiotherapy in a country such as Georgia, while Jamie O'Hara (CEO of HCD Economics, UK) addressed how joint management contributes to better quality of life in the comprehensive care of people with haemophilia, and possibly reduces costs as well.

Merel Timmer (Van Creveldkliniek University Medical Centre Utrecht, The Netherlands) and David Stephensen (Kent Haemophilia and Thrombosis Centre, UK, and Haemophilia Centre, Royal London Hospital, UK) concluded the symposium by discussing how novel uses of technology, such as mobile apps and self-administered ultrasound imaging, can be used by physiotherapists to empower patients and give them the chance to manage their own care.

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Building on our *Heritage* to Pioneer a *New Era* of care

**Pfizer** Haemophilia

## Global commitment to patients through community initiatives

At Pfizer, we are proud of our heritage of innovation through collaboration and partnerships in haemophilia care. Through working with the haemophilia community and as part of our ongoing commitment to the field, we have been proud to support the following patient / physician tools and educational activities in 2018:



In 2018, we launched the **Ready Rufus** board game, developed with leading educational gaming experts, healthcare professionals and patients for children 6–10 years old, with mild, moderate and severe haemophilia. We hope it helps kids, adults, friends and family learn more about haemophilia in a fun and interesting way.<sup>8</sup>



The **Haemoassist<sup>®</sup> 2** patient app is a smartphone app and website designed as an advanced alternative to paper-based diaries, enabling reporting and documentation of bleeds and factor infusions at the touch of a button. The Haemoassist<sup>®</sup> 2 tool supports people with haemophilia regardless of their choice of dotting factor concentrate.<sup>9</sup>



**Physiotherapy School for Haemophilia** is an educational programme that has been running since 2016. Through collaboration with an expert physiotherapy team, our goal has been to advance the science and understanding of the role of physiotherapy in improving outcomes for people with haemophilia.<sup>4</sup>



With over 600 healthcare professionals trained over the last 6 years, the **Pfizer Ultrasound Programme** aims to support initiatives to provide education, awareness, research support and hands-on training in the use of point-of-care ultrasound in people with haemophilia around the globe.<sup>4</sup>

In 2018, Pfizer continued to support the European Haemophilia Consortium (EHC) by contributing funding towards the following EHC-organised activities:<sup>5</sup>



<sup>8</sup>Ready Rufus was initiated and funded by Pfizer as part of their mission to change the world for people with haemophilia. The game was designed in collaboration with experts in gaming and healthcare, and with the patients it is intended to serve. Ready Rufus is currently only available in the UK.

<sup>9</sup>StarConsult is the legal manufacturer of Haemoassist<sup>®</sup> 2 and Pfizer holds sole distribution rights. Haemoassist<sup>®</sup> 2 is a CE-marked medical device in Europe, currently available in Germany and Spain, with planned additional launches in 2019. It is currently neither registered nor available in other regions. Haemoassist<sup>®</sup> 2 is not intended for curing, treating, seeking treatment for, managing or diagnosing a specific disease or any specific health condition. Pfizer will not have access to any personal information entered into Haemoassist<sup>®</sup> 2.

<sup>4</sup>These programmes have been initiated and funded by Pfizer.

<sup>5</sup>These activities are initiated and organised by EHC. The content and activities described are independently developed by the society. Pfizer is not the sole commercial sponsor for EHC.

To find out more about our support services and tools, contact your local Pfizer team.

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*For the Community*



## *Roche's commitment to haemophilia*



Novel therapies are ushering in a new era of opportunity to redefine what it means to live life with haemophilia.

To ensure that emerging opportunities focus on what matters most to the community, at Roche, we are continuing to build lasting partnerships with patient groups through strategic collaboration projects which aim to advance haemophilia care. We're convinced that success requires listening, understanding and a transparent exchange of information.

For more information on what we do, please visit [www.roche.com/haemophilia](http://www.roche.com/haemophilia)



## *Roche's participation in the EHC PARTNERS programme*

Access to good haemophilia care is sadly a privilege rather than a standard.

The EHC PARTNERS programme is an innovative initiative whose goals closely align with our own – to improve access to treatment options and improve the quality of care for people living with haemophilia A. Through this programme, EHC creates an incentive for governments to improve haemophilia care in countries that provide little or no treatment to their haemophilia patients.

We are grateful for having been able to join this innovative programme in 2018.

*"We are proud to be part of the PARTNERS programme and look forward to collaborating with the EHC on this important initiative. Healthcare systems vary greatly across Europe and we are committed to address local healthcare needs to ensure people with haemophilia A can have access to treatments."*

**Cristin Hubbard,**  
responsible for Haemophilia at Roche

For more information on the EHC PARTNERS programme, please visit [ehc.eu/partners/](http://ehc.eu/partners/)

## ReDEFINE HAEMOPHILIA

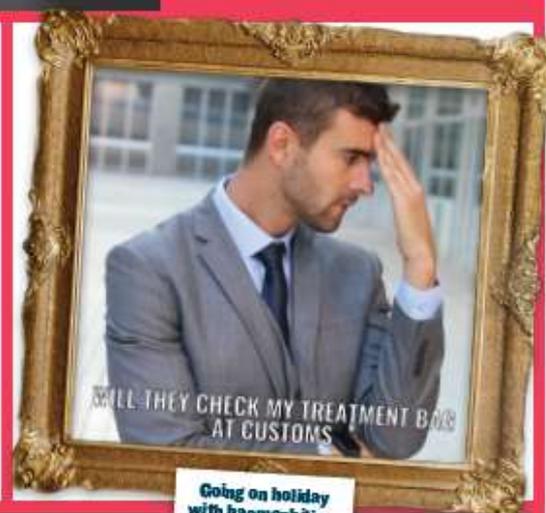


ReDefine Haemophilia is an initiative that aims to empower, spark conversation and inspire the community to redefine what it really means to live life with haemophilia and demand for more from life. It provides a unique platform for people to communicate about haemophilia in their own way, by sharing personal experiences and connecting with, learning from, and educating others.

### REDEFINE AT THE EHC ANNUAL CONGRESS 2018

We had the privilege to participate in the EHC Annual Conference 2018 in Brussels, Belgium. It was a fantastic opportunity for our team to meet and engage in interesting and insightful conversations, be inspired, listen to the community, and share a common vision for the future of haemophilia.

At our booth, we introduced #memeophilia – a meme-generating ReDefine initiative which allows members of the community to create and share memes that redefine what matters most to them. Take a look at some of the examples created below:



Going on holiday with haemophilia...



Meeting someone else with haemophilia...



Coping with joint pain and brading...



Speaking to a doctor...

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

**LET'S CONTINUE TO REDEFINE HAEMOPHILIA TOGETHER**

# Working together for a better tomorrow

## Committed to providing sustainable access

At Sobi, we are committed to providing sustainable access to treatment for all people with haemophilia, no matter where they live. That is why we are proud to work with partners such as the European Haemophilia Consortium to achieve this vision.

Every person with haemophilia should have an equal right to treatment that can liberate their lives and lead to a better tomorrow. People in different countries may face different challenges. But at Sobi, we refuse to accept the status quo, so we work with our partners to solve these challenges all around the world.

Our aim in each country is the same: to provide timely and sustainable access to transformative treatment for every person with haemophilia.

This requires using a mix of approaches, with different partners from the haemophilia community, to address specific needs in different countries.

- 1) **Developed countries** - Wealthier countries usually have developed healthcare systems and the financial resources to fund sustainable, commercial access to treatment. In such countries, we work to provide people living with haemophilia with access to treatments that liberate them to live rich, full lives.
- 2) **Partly developed countries** - Some countries have functioning healthcare systems but lack the financial resources to provide adequate levels of treatment for people with haemophilia. We are part of the EHC's PARTNERS programme, which aims to increase access to replacement therapies in European countries that provide little or no such treatment to their haemophilia patients.
- 3) **Developing countries** - To help in developing countries, which lack both functioning healthcare systems and the financial resources to provide care and treatment, we are partnering with Bioverativ, a Sanofi company, to donate 1 billion international units of extended half-life clotting factor treatment over a period of 10 years. As part of this, we are working with the World Federation of Hemophilia's Humanitarian Aid Program, to provide predictable access to treatment where it was never possible before, as well as supporting the expansion of local infrastructure and competence to help people with haemophilia. This programme has so far helped more than 16,000 people in more than 40 countries.<sup>1</sup>

<sup>1</sup><https://www.wfh.org/en/humanitarian-aid-program>



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## Youth voices vital for the future

Strong youth voices are vital for the future success of organisations such as the EHC and its national member organisations. That is why we in 2018 donated our symposium space at the EHC Conference to the annual Youth Debate.

The Youth Debate is an important milestone every year in the EHC's efforts of empowering young members of the haemophilia community. The youth debaters take on experienced advocates in discussions that are always full of energy, enthusiasm and leadership potential.

### Sobi, EHC ride into Brussels

A team of Sobi cyclists rode 390 km, from Mainz in Germany to the EHC Conference in Brussels, Belgium. For the last leg of the ride, from Leuven to Brussels, the team were joined by representatives from the EHC and its national member organisations.

This was the third Sobi charity bike ride to raise funds for the EHC. The funds are used to support projects for increased physical activity and an active lifestyle for people with haemophilia. As well as raising funds, the bike ride aims to raise awareness about haemophilia and how people with haemophilia do not need to be limited by their condition.



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## Putting hemophilia patients first

*yesterday, today, tomorrow*

At Bayer, we're all about helping people with hemophilia to break boundaries, thrive and achieve their full potential.

We've been doing this for nearly 30 years and are proud to work with patients, caregivers and treaters to help create a strong community dedicated to reaching these goals.

But we never stop.

Our portfolio of innovative recombinant FVIII therapies aims to set new standards of care. And, our evolving patient support programs and tireless research into gene therapy and bleeding disorders are helping redefine what it means to live with hemophilia.

**Breaking boundaries in hemophilia**

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01/2019

## Driven by Our Patients

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



**CSL Behring**

## Spotlight on The Novo Nordisk Haemophilia Foundation (NNHF)

For more information, please see <http://www.nnhf.org/>

The Novo Nordisk Haemophilia Foundation (NNHF) was founded in 2005 by Novo Nordisk, a global Danish pharmaceutical company. NNHF expresses Novo Nordisk's commitment to corporate social responsibility.

Realising that vast differences in care and treatment standards exist, depending on where concerned people live, was the starting point of the company's considerations. Moving haemophilia and allied bleeding disorders higher on the global healthcare agenda and providing access to care are key elements of Novo Nordisk's approach to impacting change in disease areas in which it can make a difference as a company and good corporate citizen.

Based in Zurich, Switzerland, NNHF receives approximately CHF 2.8 million per year from its main benefactor, Novo Nordisk. Other benefactors are permitted by the foundation's Council.

One of the Latest Project approved in November 2018: Albania

### Partner institution:

University Hospital Centre (UHC) "Mother Tereza", Tirana

### Represented by:

Dr Adela Perolla, haematologist and Dr Bledi Kalaja, haematologist

### Duration:

2 years, envisaged start in Q1 2019

### Status:

Formalisation

### Objectives



- Strengthen multidisciplinary care at the haemophilia centre in Tirana including specialised training of 4 healthcare professionals
- Raise awareness of haemophilia amongst healthcare professionals across Albania
- Empower the patient organisation and community through workshops
- Expand diagnosis capability at the haemophilia centre in Tirana to include other coagulation disorders
- Increase positioning of haemophilia in the health agenda through strategic media engagement and regular meetings with authorities

For a complete list of the Foundation's project, please see the NNHF.org website

A new name has joined the bleeding disorder community in 2019: Takeda has completed the acquisition of Shire, the rare disease company which had taken over Baxalta (a spin off from Baxter) in 2016. We ask Wolfhard Erdlenbruch, Takeda's Global Head of Medical Affairs Haematology, to shed light on the company's vision for persons with inherited bleeding disorders. *Text: Tineke Zuurbier, Takeda*

#### What can we expect from Takeda?

You can expect that we continue to be guided by the principle that *every bleed matters* for persons with bleeding disorders. For persons with haemophilia, our "north star" is healthy joints and quality of life. For persons with Von Willebrand's Disease, especially the rarer types of VWD, we believe that people should obtain a timely diagnosis and treatment tailored to their needs. Same applies to rare coagulation disorders like FVII deficiency.



#### How does Takeda expect to get there?

To achieve this goal, we partner with patient organizations, physicians, caregivers and researchers as well as with access related stakeholders, to understand the unmet need and address local barriers to care. In the spirit of this multi-stakeholder dialogue, we are pleased to have supported the European Haemophilia Consortium's Roundtables, Youth Workshop, Leadership Conference, Annual Conference and Inhibitor Summit over the years, many of these projects since their inception.

#### What are you concretely doing for the bleeding disorders community to achieve your goals?

We continue to optimize access and invest in innovation. As a hemophilia community we don't know enough about the *Why* and *When* hemophilia patients experience bleeds and what predicts the developments of inhibitors, amongst others. Like a fingerprint, each patient's experience is unique and requires a personal approach, based on individual pharmaco-kinetic and activity profiles, so our research focuses on unraveling the individual patient experience. For hemophilia, that means we continue to research pharmacokinetic-based dosing, gene therapy, our bi-specific antibody and oral administration.

#### Is hemophilia your sole focus?

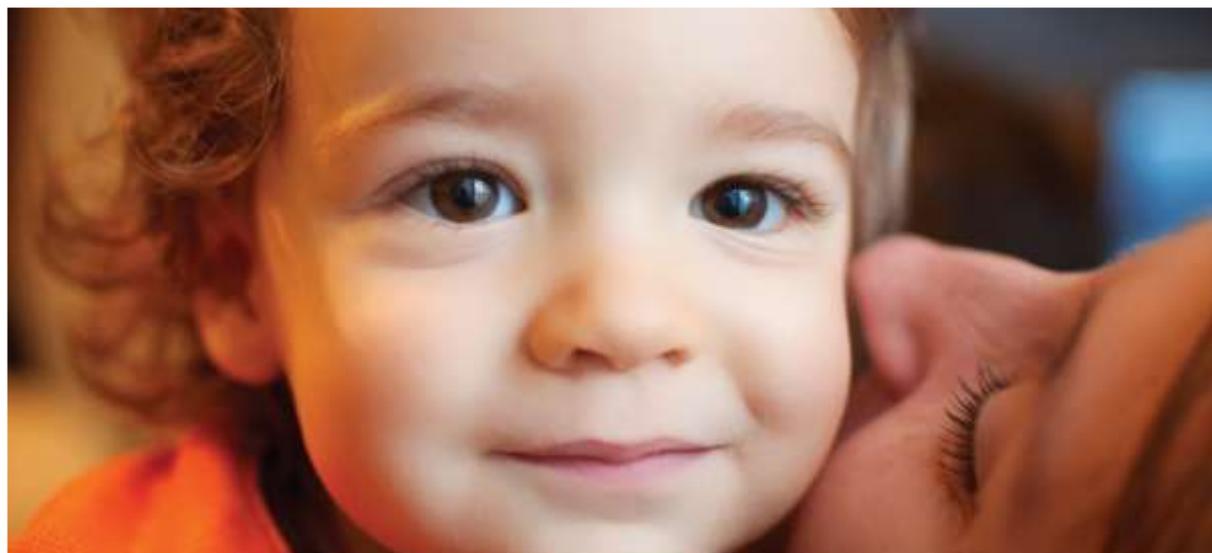
Takeda focuses on rare diseases, oncology, immunology, gastroenterology and neuroscience, and hematology, amongst others. In addition to hemophilia, we are active in a broad range of hemostasis related fields like VWD, FVII deficiency, Acquired Hemophilia, protein C deficiency, and we are expanding into other areas of rare hematology which are facing significant challenges for treatment. Stay tuned for updates when data becomes available, for example through the EHC's periodic review on "Novel Treatments".

#### Are you involving patient groups or patient representation in your research?

In 2018 at the EHC's Leadership Conference, the EHC and many of its national member organisations expressed concern that patients or patient groups are invited at too late a stage. So we need to work closely with patient groups to get better at this. We at Takeda try to do: for example, we have established a multi-stakeholder collaboration in Ireland with the Irish Haemophilia Society, the Science Foundation Ireland and researchers at the Royal College of Surgeons and other hospitals known as the IPATH study, to understand more about the relation between individual patient characteristics and bleeding risk, joint disease, and inhibitor development.

#### This being an "end of year" newsletter, what was the highlight of 2018?

There are many! One highlight was the joint statements that EHC published with the EAHAD, including the "European principles of inhibitor management". The Annual Conference where we hosted the "Every Bleed Matters" symposium was another highlight for us. We look forward to continuing fruitful partnerships with the EHC and its NMOs in 2019!



## Science matters. Because patients matter.

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## Creating the path for advancements in haemophilia gene therapy research

*"It is incredible to think of what a transformative advance gene therapy could be – it has been my career focus for the past two decades to solve this challenge."*

Katherine A. High, M.D., President and Chief Scientific Officer



**Spark**   
THERAPEUTICS

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We recognise that the path to transforming the lives of patients takes curiosity, courage and drive. The resilience of the people we serve is our inspiration to push through barriers to success. With your continued support, Spark Therapeutics is striving to challenge the inevitability of genetic disease by discovering, developing and delivering treatments in ways unimaginable – until now.

**LEARN MORE:**

Contact [patients@sparktx.com](mailto:patients@sparktx.com) for more information.



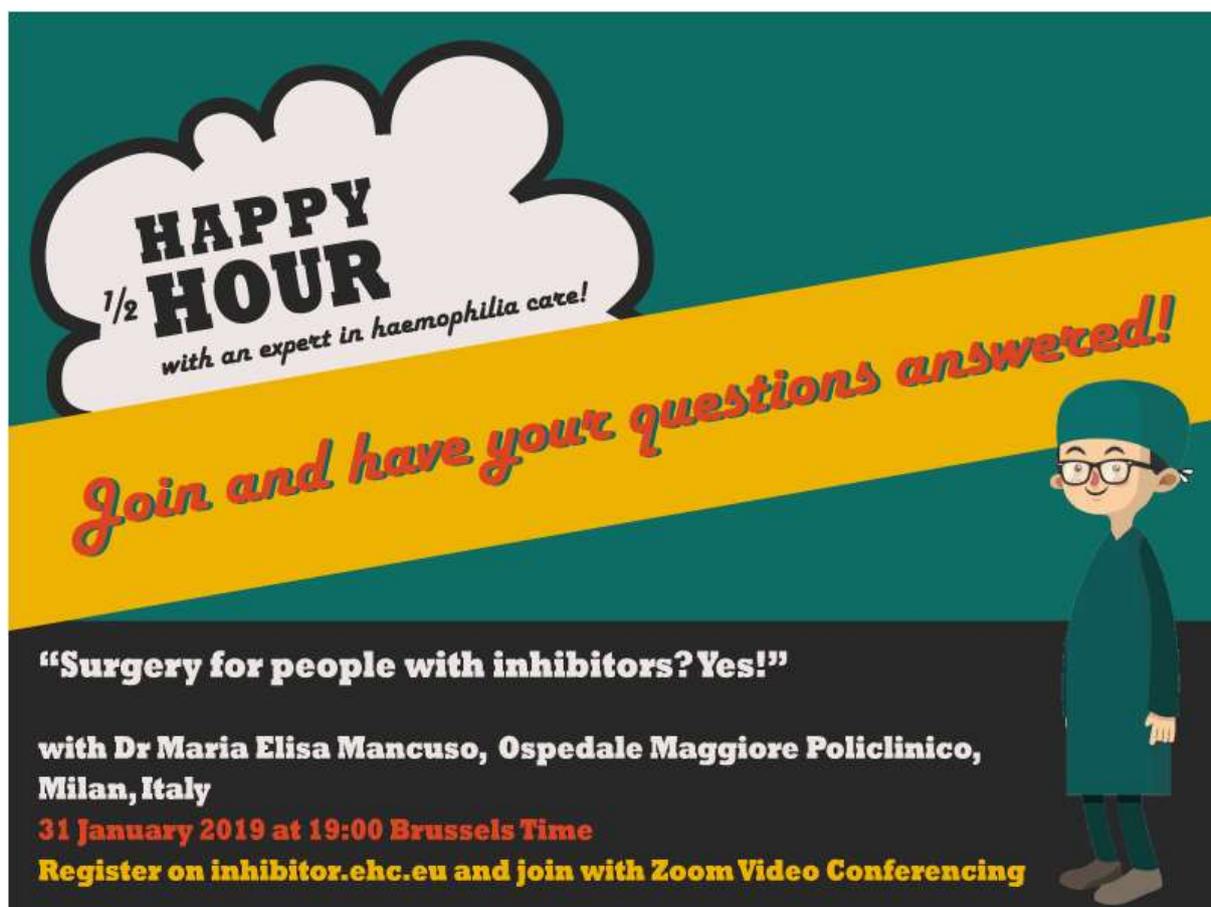
## CALENDAR OF EVENTS

### EHC 2019 EVENTS

February 20	Round Table of Stakeholders on 'The future of comprehensive care and outcomes' <i>Brussels, Belgium</i>
April 5-7	Youth Leadership Workshop <i>Amsterdam, the Netherlands</i>
April 16	World Haemophilia Day 'Celebrating 30 Years of EHC: 1989-2019' <i>Dublin, Ireland</i>
May 24-26	First European Conference on Women and Rare Bleeding Disorders <i>Frankfurt, Germany</i>
June 11	Round Table of Stakeholders on 'Pain Management in Haemophilia' <i>Brussels, Belgium</i>
June 20-23	Leadership Conference <i>Brussels, Belgium</i>
September 6-8	Workshop on Tenders and Procurement <i>Location TBC</i>
October 3	Pre-Conference Workshop on the European Inhibitor Network <i>Skopje, Macedonia</i>
October 4	Annual General Assembly <i>Skopje, Macedonia</i>
October 4-6	2019 Conference <i>Skopje, Macedonia</i>
November	Round Table on 'Markets and Access' <i>Brussels, Belgium</i>
November	Gala Dinner '30 Years of EHC' <i>Brussels, Belgium</i>
November 22-24	Workshop on New Technologies in Haemophilia Care <i>Athens, Greece</i>
Nov 28 – Dec 1	<i>Inhibitor Summit</i> Barretstown, Ireland

[Consult all EHC events on our website](#)

## ANNOUNCEMENTS: HAPPY HALF-HOURS



**HAPPY**  
**1/2 HOUR**  
*with an expert in haemophilia care!*

*Join and have your questions answered!*

**“Surgery for people with inhibitors? Yes!”**

**with Dr Maria Elisa Mancuso, Ospedale Maggiore Policlinico,  
Milan, Italy**

**31 January 2019 at 19:00 Brussels Time**

**Register on [inhibitor.ehc.eu](http://inhibitor.ehc.eu) and join with Zoom Video Conferencing**

Join us every last Thursday of the month for an ‘Ask the expert’ session in which we will feature a health care professional discussing a particular medical topic affecting people with haemophilia and inhibitors. This is your chance to learn more about a specific medical issue and get all your questions answered.