

# EHC NOW!

## Compilation Newsletter

July 2019 – January 2020



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## Message from the President and CEO

Dear Friends,

It's a pleasure to present you with a new edition of our compilation newsletter in which we put together all the editions of EHC Now!, a weekly article focusing on one current topic in our community. In this edition, we have compiled all EHC Now! news from September 2019 to January 2020. We hope you will enjoy reading it.

### The end of an era and the beginning of a new stage in EHC's life

Much has changed in these last five months. Firstly, we have said farewell to Brian O'Mahony, who presided the EHC from 2011 to 2019. Under Brian's leadership, the EHC grew exponentially in all senses. The EHC staff grew from one part-time employee to a team of seven. The EHC extended its activities well beyond the annual conference and the Round Tables of Stakeholders. This activity was done to serve all members of our community. The EHC focused on hepatitis C, on people inhibitors, on women and bleeding disorders, and most recently on people with von Willebrand Disease. Under Brian's leadership, the EHC focused on building capacity within its members, in offering guidance on leadership and association management and in training the next generation of leaders with a robust youth programme. Brian's vision also included encouraging our members to become more active in haemophilia care decision-making. To do so, he built and delivered training in health economics and pushed for workshops to give information on developments in new treatments in haemophilia care.



*After eight years, Brian O'Mahony stepped down as EHC President in 2019*

Brian strived to improve the lives of those with rare bleeding disorders throughout Europe and so, in 2017 the EHC launched a visionary programme, the Procurement of Affordable Replacement Therapies - Network of Relevant Stakeholders (PARTNERS). Through this programme, EHC wanted to encourage national governments in countries with sub-standard treatment to provide better access to treatment and care to those affected by rare bleeding disorders. The programme continues to evolve in its delivery and has already improved treatment in targeted countries. Despite juggling many roles, Brian was always available and ready to support EHC members when in need, through national visits, letters of support and additional trainings. The EHC will sorely miss him, but as Brian himself advocates, it is vital for organisations to change so that they can evolve to an ever-changing future. The EHC, NMOs and individuals appreciate all the time Brian has dedicated, and we know that he will remain a dear friend to the EHC. During the EHC 2019 Conference, Brian gave a farewell address to EHC members in a video available on the EHC YouTube channel. We have provided the relevant links for this below.

The EHC has also said farewell to its longest-standing employee, Jo Eerens. Jo came to the EHC in 2010 and built its office from scratch as a base to support EHC activities. Jo had many roles within the EHC, including his final role as Membership Officer, in which he was EHC's members' contact point to EHC staff and Steering Committee. Jo's kindness and generous spirit made him a perfect fit for this job, and his patience and empathy allowed him to facilitate conversations and mediate difficult situations. Jo was also our institutional memory and a direct link to the patient community. He had an incredible wealth of knowledge of EHC activities and its members but also of what the patient community had gone through in the last forty years. Jo was able always to advise his colleagues and give them a patient's perspective on any issues they faced. He will also be sorely missed. However, he generously offered to volunteer for some of our most significant events ahead and that comforts us in knowing that we will continue to see him and have the opportunity to seek his valuable advice.

Endings bring new beginnings and so the EHC General Assembly had to decide who would be guiding them in the next four years. It was our absolute pleasure to have three stellar candidates compete for the position of President. We wish to give a warm thanks to Olivia Romero-Lux (France) and Baiba Ziemele (Latvia) for putting forward their candidatures and believing in the EHC. In the end, the General Assembly put its trust in Declan Noone, a long-standing figure of the community. Declan has been a member of the Irish Haemophilia Society (IHS) all of his life, and he worked there as an employee for eight years. He left his position to complete an MSc. in Health Economics and then joined the EHC as PARTNERS consultant in 2017, a role he gave up in September 2019 in order to run for election. In Declan's Presidency he will focus on access to treatment and novel therapies into the future, as well as improvements in comprehensive care for all patients with all inherited bleeding disorders.



*Declan Noone was elected as EHC President in October 2019*

In this past few months, we also welcomed two new EHC staff members, Thierry and Nastassia, and re-allocated the membership officer roles to Kristine and Fiona. You can read all about these changes in the newsletter here below.

Furthermore, we are thrilled to announce that the EHC has purchased an office in Brussels and so closes an era of re-locations and begins a new chapter of stability with a fixed base in Brussels. We will be moving in the first quarter of 2020. In line with the theme of endings and beginning, we are pleased also to announce that our World Haemophilia Day event this year will close a year of celebrations of our 30<sup>th</sup> anniversary. We will hold an event in our new offices here in Brussels on 24 April, and during this event, we will unveil an interactive exhibition on living with bleeding disorders through the ages, to be experienced through the eyes of our diverse community!

Finally, this will be our last compilation newsletter as we decide to move to a shorter and more manageable news cycle. We will continue to publish EHC Now! every Wednesday on our website and social media channels but from February 2020 onwards, we will email you a monthly electronic newsletter collecting all EHC Now! shared during the month and also keep you informed of other EHC activities. We are hoping that this new system will make it easier to stay abreast of EHC news and activities, and look forward to your feedback!

### **Looking ahead in 2020**

You will find a calendar of events at the end of this newsletter; however, we would like to give you a quick snapshot of what 2020 will hold in store for our community. First of all, we will develop a network for people affected by von Willebrand Disease. We have launched a call for volunteers, and if you would like to get involved and shape our activities on this topic, you have until 14 February to apply.

Following a successful edition of our Women and Bleeding Disorders Conference in 2019, we will hold a second such conference in May in Basel. Some of the themes of this conference will be similar to last year, but we will focus on new issues such as ageing and musculoskeletal health. We have also built in a lot of 'open time' with experts so that each 'disorder group' can discuss specific issues. Finally, we will embark on a journey on how to deal with family relations and dating. It will be exciting, and we cannot wait to be there! We hope you will be there too!

The Youth Fellowship programme has begun to bear its fruits, and we have had a first camp organised by the WISH project (read more below). We are now proud to announce another project, an international football camp for people with bleeding disorders. This will be held in the summer in the Netherlands. Read below for more information on this. This was inspired by the long-standing

basketball camp organised by our Lithuanian NMO – it is great to see our community inspire each other across borders.



*Amanda Bok is the EHC CEO*

Another year, another documentary to capture your important stories and shine a light on the path ahead. Our next documentary will focus on ageing with a bleeding disorder. A wonderful development in our community, but uncharted and not without its many challenges. So please stay tuned and help us disseminate it widely when it comes out (in the autumn).

Last but not least. We're diving into the world of technology and currently working on developing an educational app for our community, intended to provide tailored education for all patients, adapted to different

learning styles and levels, on treatment and care. It's ambitious but necessary and we can't wait to tell you more, hopefully soon!

2020 will be a big year, for all of the above, and also because it's the year when gene therapy in haemophilia is very likely to be licensed in Europe. Only time will tell how this will impact the status quo in haemophilia care in Europe; however, it is safe to say that the future is now, and we're working hard, collectively, to make sure it's a positive one for all members of our community.

We hope you will enjoy the newsletter and please do not hesitate to get in touch with the EHC office, for any additional information.

## EHC News

### EHC elects Declan Noone as its new President

*On Friday 4 October, the General Assembly of the European Haemophilia Consortium (EHC) elected a new President, Mr Declan Noone. He replaces Mr Brian O'Mahony, who served for eight years as EHC President and 12 years as Steering Committee member.*

We spoke to Declan during the EHC 2019 Conference, shortly after his election, and he gave us the following statement:



*Declan Noone congratulated by the community, minutes after his election*

*"I would like to thank the community for this incredible honour of becoming EHC President. In the last thirty years, I saw as an NMO member at first and subsequently as a volunteer and staff member, the community growing and strengthening in terms of their advocacy efforts. I am now so honoured that the community has given me their confidence in leading them into the next four years. This weekend has been a whirlwind. I have had the opportunity to meet with a lot of EHC members and learn about their priorities, and I was delighted to see their hope for a better future. I want to help each NMO in becoming their best advocates and getting access to the treatment they deserve.*

*I also wish to thank Brian O'Mahony for the work he has done in the past eight years. I've had the pleasure to work with Brian for over a decade, and I greatly admire his leadership, vision and commitment towards the community. I am stepping in big shoes. The bar has been raised in terms of what the community will expect from their president. I want to assure them all that I am feeling ready to take on this challenge.*

*As I said in my candidature speech, I want to serve all communities such people with inhibitors, ageing patients, women with bleeding disorders and any patient in need of help in getting access to the treatment and care they deserve for a better quality of life."*

Mr Declan Noone is the former EHC PARTNERS programme consultant. He held this position since 2016 and until September 2019. Prior to this, Declan worked at the Irish Haemophilia Society (IHS), when he was also a member of the Irish Haemophilia Product Selection Monitoring Advisory Board (HPSMAB). This body was in charge of product selection used in Ireland to treat haemophilia and other rare bleeding disorders. He also worked in the area of hepatitis C and access to treatment.

Declan was a member of the World Federation of Hemophilia (WFH) and sat on several committees such as the Educational Materials Committee, the Data and Demographics Committee and the Safety and Supply Committee.

Declan co-authored papers on the quality of life in people with haemophilia. He also worked on surveys on access to haemophilia care and procurement methods in Europe. He was an author of the WFH Monograph entitled "An Introduction to Key Concepts in Health Economics for Haemophilia." Recently he published on European procurement methods. Mr Noone has an M.Eng, in the Medical Mechanical Engineering and an M.Sc in Health Economics.

Mr Brian O'Mahony addressed and thanked the community for its support in a [short video available online](#).



*The old and new guard: The EHC Steering Committee together with former (Brian O'Mahony - third from left), and newly elected EHC President (Mr Declan Noone - fourth from left). Ms Naja Skouw-Rasmussen, EHC Steering Committee member is not pictured.*

## EHC welcomes two new staff members on the EHC team

*In the past six months, two new staff members have joined the EHC team. Here below we give you a quick interview with Thierry Hoppe, EHC's Office Administrator and Nastassia Kisialeva, EHC's Communications and Public Policy Officer.*

### **Introducing Thierry Hoppe**

*In June 2019, the EHC team grew by one extra member by welcoming Thierry Hoppe who becomes the first EHC Office Administrator. In this position, Thierry helps the EHC to manage its finances and human resources. In this article, we interview Thierry to learn a little bit more about him and his impressions on the EHC Workshop on Tenders and Procurement, which he attended in September 2019 in Istanbul.*

**Thierry, when we first met, you described yourself as a finance and HR manager, having worked on accounting, budgets, forecasts and payroll. So, I take it, you like numbers – can you explain why, and what you like about your line of work?**

Indeed, I prefer figures and numbers to words! I find numbers more rational and easier to understand. They clearly show reality. Words, on the other hand, are more versatile, they can be misconstrued and interpreted differently depending on the people who say or hear them and situations in which they are used.

I also like the fact that finances and accounting reflect what is going on in an office. They give a very clear picture of what we're working, how work is organised and the results achieved. They can also indicate how office life should be organised.

**For the last 12 years you were on the management team of the European Cancer Organisation (ECCO) – can you tell us a little bit about ECCO for those who of us who don't know it?**

ECCO is an umbrella organisation representing associations of doctors treating cancers, that are organised either by the organ impacted by cancer or by medical speciality. I worked there for 12 years in a similar role. The main differences with EHC are that ECCO represented clinicians while EHC represents patients and also the size of the organisation. We had a staff of around 40 people. Also, everyone knows cancer and, sadly, most people are touched in one way or another by cancer. One of our main activities there was to hold a yearly congress, which gathered thousands of specialists from across Europe and internationally.



*Thierry Hoppe (centre) during the EHC 2019 Leadership Conference*

**At ECCO, you worked with volunteers – we have this in common. What is your take on the volunteer sector and what was your experience with volunteers in your previous job?**

As I mentioned above, ECCO was an organisation representing doctors, and so our board was composed of clinicians volunteering within the organisation. They were the middle-man between the general assembly and staff. They helped in steering the organisation's activities. However, unlike EHC, we didn't have as much contact with our members seem as the main activity was our congress. We didn't have so many working groups and committees or programmes. So far, and I've only been here for a few months, I must say that it has been quite enriching meeting patient representatives from across Europe.

**A few more personal things. You are a French-speaking Belgian from the city of Liège. What can you share with our community about your native city?**

I come from Liège, it's a border city in the south-eastern part of Belgium, and it's very close to both Aachen in Germany and to Maastricht in the Netherlands. It's also a university city, which gives it a very dynamic and young vibe. They say that people from my home town are quite proud and see themselves as a little detached from the rest of Belgium. However, I have been living in Brussels for over 20 years now. This is where I raised my family.

**At EHC staff level we're very curious, can you tell us a little bit more about your hobbies and what you do in your free time?**

I have two hobbies; one of these is cycling. I cycle most weekends, either in a group or alone. I particularly like this sport because it is both a team sport but also you are cycling alone. I've been cycling now for over ten years and last summer I crossed the French alps by bike.

My other hobby is more something I did for fun more than anything else. Some friends started a small start-up in which they buy grapes and make wine here in Brussels. It's something that is already done in other cities, but not in Brussels. I have a small share in this enterprise and am proud to be supporting Brussels wine-making. This hobby is quite recent, though, and we will see if it leads anywhere.

**So, you're a married man and have three children. Is there anything you can share about your journey as a parent?**

I have three children in their 20s now, and I must say that everything went smooth, raising them. I am not sure that I can give you much advice because I think my wife and I are very fortunate with our kids! However, if I had to give one piece of advice to any parents, it would be to make sure that you treat

each of your children as their own person and that you give them age-appropriate freedom and autonomy, so they have a margin to test new things and make errors.

**Before starting work at EHC, did you have any preconceptions about either haemophilia or patient organisations? If so, how have these changed since you started?**

I did not have any direct contact with patient organisations before this job. In the cancer space, there are many patient organisations, but they usually organise themselves around the type of cancer or the organ impacted, so there's a bit of fragmentation. Although EHC represents more than just haemophilia, I feel that there is more unity within the community. I also find the work the EHC carries out very constructive, and I like that it doesn't just analyse the problem but comes up with concrete actions and solutions. I like that I get to work on numbers but that there is a very human face to them and that I can support a worthwhile cause.

**On 6-8 September, the EHC held a workshop on Tenders and Procurement in Istanbul, Turkey. The workshop was aimed at central European countries and starts a new series of these workshops as it looks at evaluating haemophilia treatment, including non-replacement therapies. This workshop was the second EHC event you attended. What were your impressions of the weekend?**



*Participants at the 2020 EHC Workshop on Tenders and Procurement. Thierry is first row, third from the left.*

To be very honest with you, I didn't really know what to expect. The title of the workshop didn't mean much to me and the weekend was an eye-opener. I had no idea how haemophilia treatments were procured, and I realised even less that patients could play such an essential role in the procurement process! I liked that the workshop lectures gave general principles that can be tailored to each country's needs and organisational structure. I also really see how it can be quite tricky for national organisations to find people who will have the time and interest to develop skills and expertise in health economics and procurement processes. It's very complicated, but it's so worthwhile. I like that the EHC gives out tools and education in this area. I particularly liked the group exercises carried out during the weekend. It was interesting to see what participants consider to be of importance in the treatment product purchased. I also really enjoyed the session on case-studies and what is done in different countries. I must say before coming here, I had no idea that patients could take such an active role and lead advocacy efforts.

***Thanks, Thierry! We're delighted to have you on the EHC team, and I'm sure our community will soon enjoy getting to know you better, as much as we have!***



## Introducing Nastassia Kisialeva

*Nastassia Kisialeva joined the EHC team as Communications and Public Policy Officer. Nastassia or Nastia – as she's nicknamed – will work together with Laura Savini to support EHC's communications and public policy activities. As the EHC heads into a strategic retreat this weekend to look at its work for the next four years, we have a quick chat with Nastia about her background and what she is looking forward to in her new position.*

### **Nastia, you have a double nationality, Belgian and Belarussian, can you tell us a little bit more about that:**

I was born in Belarus and moved to the southern French-speaking part of Belgium with my family when I was a child, and this is where I lived and studied before moving to Brussels at the end of last year. Although I still have some relatives in Belarus, most of my immediate family is here in Belgium.

### **What is your professional background? Where did you work before coming to the EHC?**

I studied translation at university and focused on Russian, English and French. Just after graduation, I worked for a consulting firm. My main job there was to organise training for veterinaries from across Europe on the latest European veterinary legislation. These events were part of a European-funded project. We took care of all of the logistics and content. After a couple of years, I moved to the Belgian Ministry of Defence and became a translator and analyst. My job there was to do translations from English and Russian into French. I worked closely with the military as a civilian, and that gave me some exciting insights into the working of the Belgian government. I stayed in this position for five years, and although the job was rewarding, I felt that I needed a change.



*Nastia joined the EHC team in January 2020*

### **You have no previous experience with patient organisations or haemophilia, what attracted you to the position?**

While working at the Ministry of Defence, I felt that I lacked contact with people influenced by my work. I wanted to find something where I could see the concrete impact of my day-to-day work and contribute to valuable projects, as the ones implemented by EHC. This is why EHC appealed to me and made me decide to apply for the role. I like the idea that as part of the EHC staff, we are in touch with National Member Organisations (NMOs) and get to hear first-hand about their issues and then work together to identify ways to overcome those issues.

### **What will be your priorities in the first few months in the job?**

I am new to this community, and the first thing will be to get up to speed on content and getting a better grasp of the environment in which the EHC operates. It is my understanding that the EHC is always evolving, and new activities are continuously emerging, as you will have seen from yesterday's post in which we announce the creation of a Von Willebrand Network!

As noted above, I will be working closely with Laura on Communication and Public Policy activities. The exact plans of how those tasks will be shared between us are still in the works, and probably a lot of it will be discussed during the strategic retreat this weekend. For now, though, I can already say that I will take over the organisation of the Round Table of Stakeholders and the organisation of the World Haemophilia Day event that will be held on 24 April here in Brussels.

Another big part of my job will be to oversee the translation of our website into Russian and to ensure timely communication with our Russian-speaking members. As readers probably know by now, Jo Eerens, the former EHC Membership Officer, retired in November, and the EHC is now considering how to share his tasks best. I won't say much more though as this is something that we will announce in next week's EHC Now!

On a personal note, though, I heard that there are still some significant discrepancies in terms of access to care between Western and Eastern European countries and I want to put my language skills to contribute to support the work of that part of the community.

**Since you bring pretty impressive language skills to the job, we're introducing our 'Russian Friday' on social media, can you tell us what that is?**

EHC was set-up to serve its members both in English and in Russian, and for years, the EHC wasn't able to do this as much as it would have liked. I want to use my language skills to contribute further to serve these NMOs, and one first step in doing this is to give them more timely information. So, we decided that our social media posting (on Facebook and Twitter) each Friday will be a summary of the week's news and updates in Russian. In this way, our members will have a regular and quick way to see what the EHC and the community at large have been up to. Keep an eye for it on our social media: the first edition is coming this Friday.

**Finally, to conclude the interview, can you tell us about you on a more personal level. What do you enjoy doing in your free time?**

I love spending time with friends and meeting new people. I also love to read, go to the theatre and concerts and doing some sport. I generally enjoy having a good time with my partner, friends and loved ones.

*Thanks, Nastia! We're delighted to have you on the EHC team, and I'm sure our community will soon enjoy getting to know you better, as much as we have!*

## **Updates on community, communication and grants for NMO's**

With quite a few recent changes in the EHC staffing, the optimal communication with the EHC National Member Organisations (NMOs) has been reviewed. We are delighted to bring you updates in membership matters, communication to the NMOs, as well as a few good reminders about the EHC event calendar!

**This article will provide information on the following items:**

- Membership Officer
- Events
- Travel Grants
- Activity & Physical Activity Grants
- Committees
- Articles from our Members

**Membership Officer**



It is very important to the EHC to maintain close relationships with our National Member Organisations (NMOs) and for our NMOs to reach out to us for advice, support and information. The role of Membership Officer formerly held by Jo has now been reassigned to [Ms Fiona Brennan](#) and [Ms Kristine Jansone](#).

*Jo Eerens recently retired as EHC  
Membership Officer*

Furthermore, for the past number of years, Steering Committee members have been assigned as points of contact to different NMOs. To further facilitate this process from January 2020, we have introduced a tandem approach where a Steering Committee member will also be paired with a staff person from the EHC office to support and best serve the interests of our NMOs through regular communication! For information on your NMOs points of contact, please consult the table below:

Steering Committee – Staff member – NMO Liaison		
NMO	SC Contact person	Staff member liaison person
Austria, Belgium, Cyprus, France, Germany, Italy, Luxembourg, Slovenia and Switzerland	Olivia Romero-Lux	Laura Savini
Albania, Bosnia and Herzegovina, Bulgaria, Croatia, Greece, Montenegro, North Macedonia, Portugal, Serbia and Spain	Miguel Crato	Saskia Pfeyffer
Denmark, Finland, Hungary, Iceland, Ireland, Israel, Norway, Sweden and the United Kingdom	Naja Skouw-Rasmussen	Fiona Brennan
Czechia, Estonia, Latvia, Lithuania, Moldova, Poland, Romania, Slovakia and Turkey	Michael van der Linde	Kristine Jansone
Armenia, Azerbaijan, Belarus, Georgia, Kyrgyzstan, Russian Federation, Tajikistan, Ukraine and Uzbekistan	Thomas Sannié	Nastassia Kisialeva
The Netherlands	Minette van der Ven	Saskia Pfeyffer

## **Events**

Each year the EHC organises many events for our NMOs along with many other communications distributed to our members. With our NMOs also very busy with internal activities, it is important to us that you do not miss deadlines or information regarding our events that may be of interest to you.



*EHC Members can benefit from travel grants to several events, including the EHC Round Tables of Stakeholders*

In the [events section of our website](#), you can find the information on what events are currently open for registration. There, you will find information on the event's topic, participants' eligibility and EHC staff contact. This information is to ensure that we answer any questions you may have about the programme. Finally, the EHC will send as of February, a monthly communication to our NMOs, highlighting the events currently open for registration or application.

### **Travel Grants**

The EHC want to make our events as accessible as possible for our members; we, therefore, cover the full cost of participants to attend certain EHC events. For other events not fully reimbursed by the EHC, we do provide Travel Grants to some of our NMOs. Information specific to each event and the respective point of contact is listed below:

<b>Travel grants for EHC NMO members</b>		
<b>EHC activity</b>	<b>Travel policy</b>	<b>Contact point</b>
EHC Workshops Leadership Conference Inhibitor Summit	Fully covered travel and accommodation to all NMO participants	EHC staff member responsible for the event
EHC Conference EHC Women and Bleeding Disorders Conference	Travel grant available	Conference Organisers Ltd.
EHC Round Table World Haemophilia Day event	Travel grants available	Laura Savini

### **Activity Grants**

NMOs have important work to do but don't always find the funding they need, when they need it. When this occurs, NMOs can apply to the EHC for a small amount of co-funding to:

- Initiate a required project/programme,
- Allow an already-initiated project/programme to continue,
- Any other activity as deemed appropriate by the NMO and the EHC.



#### **Which NMO activities are eligible?**

All NMO activities designed to benefit national members, that directly support the improvement of the quality of life of people living with a rare bleeding disorder in their country, are eligible for co-funding. Activities must also be in line with the EHC's overarching mission and objectives.

#### **Which NMO activities are not eligible?**

Activity grants are not long-term solutions and cannot be used for operational costs (e.g. rent, salaries).

#### **How does it work?**

NMOs can apply for an activity grant directly with the EHC office all year-round (as the need arises). All applications will be reviewed by the full Steering Committee (SC), and SC members will decide on the project by majority vote during their yearly meetings (three times per year). Preference will be given to NMOs that have not already received an activity grant in the year of application. Grants are provided for one year (12 months) and the SC will award them on a first-come, first-served basis.

#### **Who is eligible?**

Only EHC NMOs can apply for activity grants. Preference will be given to NMOs that:

- Have limited organisational funding and/or fundraising opportunities
- Are active members of the EHC (e.g. have regular communication, respond to EHC surveys, etc.)
- Are legal members of the EHC (e.g. are up-to-date with their membership fee payments)

For more information on activity grants, please get in touch with [Fiona Brennan](#). For more information on activity grants, [consult the EHC website](#).

### **Physical Activity Grants**

With the Physical Activity Grant (PAG), the EHC wants to support NMOs in organising physiotherapy and physical activities. When an NMOs wants to start up and organise such activity and needs funding for it because of lack of resources, they can apply to the EHC for co-funding to:

- Initiate a required project/programme,
- Allow an already-initiated project/programme to continue,
- Any other physio-activity as deemed appropriate by the NMO and the EHC.



### **Which NMO activities are eligible?**

All NMO activities designed to improve the physical capacity of people living with a rare bleeding disorder in their country are eligible for co-funding. Activities involving more than one NMO working together are also eligible for funding. All activities must be in line with the EHC's overarching mission and objectives.

### **Which NMO activities are not eligible?**

PAG's are not long-term solutions and cannot be used for operational costs (e.g. rent of office, salaries of NMO staff, office materials etc.).

### **How does it work?**

NMOs can apply for a PAG directly with the EHC office all year-round (as the need arises). The full Steering Committee (SC) will review all applications and make a decision by a majority vote. Preference will be given to NMOs that have not already received any activity grant in the year of application. Grants are provided for one year (12 months) and are awarded on a first-come, first-served basis.

### **Who is eligible?**

Only EHC NMOs can apply for a PAG. Preference will be given to NMOs that:

- Have limited organisational funding and/or fundraising opportunities
- Are active members of the EHC (e.g. have regular communication, respond to EHC surveys, etc.)
- Are legal members of the EHC (e.g. are up-to-date with their membership fee payments)

For more information on physical activity grants, please contact [Kristine Jansone](#). For more information on physical activity grants, [consult the EHC website](#).

### **Committees**

As you will already be aware, the work of the EHC is guided by several committees including our ***Steering Committee***, ***the Medical Advisory Group*** (MAG) and ***Medical and Scientific Advisory Group*** (MASAG) who actively advise and guide the direction of the EHC. Specific committees and working

groups are also created to work on behalf of targeted groups in our community, such as the Inhibitor Working Group, Women with Bleeding Disorders Committee and the Youth Committee. On occasion, the EHC will announce a call for volunteers to apply to join these or other groups. The call for volunteers will be shared with NMOs, along with the relevant Terms of Reference for the respective committee/working group, eligibility requirements, information on the application process and the closing date for candidatures. These will also be advertised on our website and social media channels.

Currently, the EHC has announced a call for volunteers to join the newly established [von Willebrand Disease \(VWD\) Working Group](#). For more information on this call for volunteers, please consult the EHC website. The deadline for applications is **Friday 14<sup>th</sup> February at 17.00 hrs CET**. For further information on the call for volunteers, please [get in touch with Fiona Brennan](#).

### **Articles**

The EHC is always delighted to hear about the activities and events of our members. We also feel it is important to share these experiences, stories, successes and information with our community. We would like to remind you that throughout the year, we would be delighted to receive articles from our NMOs on your events and activities that share your experiences. At any time of year, you can send this onto Laura for distribution. For more information, please contact [Laura Savini](#). We hope that this information is helpful for you and will help to facilitate our continued collaboration.

## **Be part of our team – EURO 2020: Adapted Football & Physical Activity Camp!**

As you can see, 2020 is going to be a big & busy year for the EHC with many events and activities to support, empower, educate and build lasting friendships in our community!! We take a look at our recently launched EURO 2020: Adapted Football & Physical Activity Camp! The camp will take place in **Utrecht in The Netherlands from the 1<sup>st</sup> – 5<sup>th</sup> July 2020**, offering our community to be part of a team in a safe, adapted and fun way – highlighting that no matter what your physical ability is you can get involved and stay active!



The purpose of the EURO 2020 Youth Fellowship Project is to organise a programme for people with bleeding disorders from NMOs across Europe. We want to bring together young patients to highlight the importance of **staying active** but also to demonstrate that this can be done in an **adapted way to suit all abilities and mobility levels**. The camp will have a strong ethos of adapted rules which will be the key for success to encourage people to fully participate and enjoy the experience.

The objectives of the programme are to encourage inclusive, active participation and physical activity in a fun and team-spirited way, from all patients in the bleeding disorder community in spite of any physical limitations. It is also to provide education and positive engagement on the importance of maintaining physical health while continuing to build our community for people across borders. The programme will include specialists in physiotherapy, nutrition and haematology.

### **Programme**

The programme of the EURO 2020 Workshop will consist of both informational and practical educational workshops and also include football matches and training. The formation of the football tournament will be as follows: Teams of 5 will be selected and created from registrations, the teams will then be scheduled matches throughout the camp. Before each match, there will be warm-up sessions. The first draft of the programme-at-a-glance can be consulted on [our website](#).

### **Participants**

The EURO 2020 event will be dedicated **exclusively to people with bleeding disorders over the age of 18 years**. We kindly invite each NMO to send **up to 5** individuals – participants will be accepted on a first come, first served basis and grouped into European teams.

### **Venue**

The EURO 2020 Programme will take place in Utrecht, The Netherlands. It is important to note that the hotel accommodation will be shared. A request to share with particular people will be facilitated where possible.

### **Registration**

**The registration to this event is now open and will close on Friday 28<sup>th</sup> February. The registration form can be found [here](#).**

It is **essential to register each delegate separately**. The delegates can note within the form the names of their peers attending to enable EHC to assign accommodation correctly.

### **Costs**

The EHC will cover all the travel and accommodation costs of all the delegates. These expenses will be reimbursed according to the EHC travel reimbursement policy.

If you have any further questions regarding the programme or nominations, do not hesitate to contact [Fiona Brennan](#) in the EHC office.

**We very much look forward to welcoming your participants to this event!**

*The Youth Fellowship Programme is made possible thanks to the sponsorship of Sanofi and BioMarin.*

## **EHC Workshop on Novel Technologies in Haemophilia Care**

*In November, some 70 delegates from across Europe gathered in sunny Athens to hear all about the latest advances in new treatments in haemophilia. This was the seventh edition of the workshop and the most attended, which underscores the growing interest and educational need.*



This workshop is one of several activities developed by the EHC to try to bring more knowledge to its community about innovative treatments. The main difference between this and other EHC events, however, is the workshop's 'community-only' tradition. This is to ensure complete freedom for patients and healthcare professionals to ask any question or raise any point they wish to, under Chatham House Rules. This means that participants are free to use the information received, but neither the identity nor the affiliation of participants may be revealed.

This year's workshop was divided into three parts: licensed therapies, therapies under development and gene therapy.

In the licensed therapies part, participants heard presentations on the clinical use of emicizumab in patients with inhibitors, considerations on when and how to switch patients without inhibitors to emicizumab, presentations on longer-acting replacement therapies, and safety and economic considerations on novel therapies. Participants also heard an update on therapies for von Willebrand Disease. In the 'therapies under-development' section, participants were given an update on anti-TFPI and SerpinPC. In the session on 'gene therapy,' besides receiving an update on the status of the clinical trials, speakers covered potential re-treatment with vector and explored thus-far unanswered questions regarding adeno-associated viruses. Ethical considerations regarding gene therapy in children and how we will be able to afford this novel therapy were also discussed. Both days featured panel discussions in which patients and clinicians were able to discuss treatment strategies using novel therapies and any other unanswered questions for the day.

Besides offering an opportunity for learning and keeping abreast with the latest developments in treatments, the workshop also shed light on the 'hot topics' within the community. This year, participants focused on the current lack of long-term data on treatment use with novel therapies. It seems that clinicians, just like patients, are wrapping their head around all of the treatment options that will be available and how to assess which treatment will better be suited to which patient. This leads to the conclusion that there will be an even greater need for personalization of care depending on the patient's family history, lifestyle and treatment goals. Another discussion item regarded how to maintain links with patients who might feel that they no longer need their treatment centre or NMO as much, and the responsibility of that NMO or Centre to manage good education and communication around these issues.

*All in all, the weekend was packed with lots of very interesting and thought-provoking discussions, and we can only but recommend to all of our members to keep an eye on the 2020 edition, which will take place from 13 to 15 November.*

*For more information about new technologies, please consult our latest [New Products Newsletters](#) and [videos on gene therapy](#).*

*This workshop was kindly sponsored by BioMarin.*

## Some more EHC news

*In the past few years, the EHC has tried to catch up with the latest communication trends and decided to start developing some shorter and catchier videos instead of writing lengthy articles. We want to include a short snapshot of these videos in our newsletter because we want to draw your attention to other activities carried out by the EHC.*



### **EHC welcomes new member during 2019 General Assembly**

During the 2019 EHC General Assembly, the EHC NMOs voted to welcome a new member from Uzbekistan. During the conference, EHC staff interviewed their president, Lilya Zinikova about their organisation and activities. You can watch the [full video in Russia with English subtitles on the EHC YouTube channel](#).

### **Dental training workshop**

During our 2020 Conference, the EHC organised a dental workshop for dentists from the Balkan region. The dental workshop was the brainchild of Dr Alison Dougall, member of the EHC Medical and Scientific Advisory Group (MASAG), who continuously strives to raise awareness about the importance and feasibility of good dental health in the rare bleeding disorders' community. Dr Dougall has taken part in many of the EHC events with a focus on people with inhibitors and women with bleeding, and she believes that good dental hygiene is within reach for all people affected by rare bleeding disorders. During the workshop, she and other colleagues provided training for some 30 dentists from the Balkan region.



[Watch the short video](#) summarising the event and highlighting key points on dental health in people with rare bleeding disorders here.

*This workshop was kindly sponsored by Roche.*

### **EHC Youth Fellowship Programme**

One of our Youth Fellowship Programme Teams held their Workshop for Ireland, Serbia and Hungary (WISH) and they travelled to the annual Serbian Youth Camp for a weekend of education, team building and a little bit of fun along the way!

The Youth Camp was attended by over 30 participants from Serbia, together with the WISH Team consisting of Karola Diosi from Hungary, Donal Mc Cann from Ireland and Serbia's own Nikola Kumovic, and EHC staff member, Fiona Brennan. The WISH Project aims to encourage cross-national engagement from youth all over Europe, as well as to involve youth in decision making & the future direction of their NMO.

Take a look at the [video summarising the camp](#) and read more about the [Youth Fellowship Programme](#).

*The Youth Fellowship Programme is made possible thanks to the sponsorship of BioMarin and Sanofi.*

### **New Products Review**

In October, EHC published another issue of its New Product Review. This is a publication containing a wealth of information on novel therapies in rare bleeding disorders that are either under development or recently marketed. The newsletter is written and edited by a group of EHC volunteers.

The next issue of this newsletter will be coming out in the coming weeks. In the meantime, [read all previous issues here](#).

### **Thematic excerpts from the Women and Bleeding Disorders movie**

In May 2019, the EHC released its Women and Bleeding Disorders movie looking at challenges faced by women affected by bleeding disorders. In September 2019, the EHC released five short videos looking at some of the key issues faced by women with bleeding disorders: dental issues, the impact of bruising, fertility, the impact of menstruation and the importance of peer support.

[Watch the full movie and all five clips on the EHC website](#).

*The movie and clips were made in part thanks to the sponsorship of Takeda.*



## **Have you ever heard of the “Stiff ankle society?”**

*They are very popular in Denmark and were broadly talked about in the Nordic meeting that took place from 15-17 November in Copenhagen, Denmark. Also, EHC Community Programmes Officer Fiona Brennan and recently-retired Jo Eerens were privileged to attend this meeting and hear all about the activities of the NMOs! In this week’s edition of EHC Now! find out more about the Stiff-Ankle Society, the Tarzan Syndrome and more from this collaborative meeting!*

This year's Nordic Meeting was hosted by the Danish NMO – Danmarks Bløderforening – and brought together five Nordic countries; Denmark, Finland, Iceland, Norway and Sweden, that were joined for the first time by their Baltic neighbours, Estonia and Latvia. The tradition of the Nordic Meeting is to meet and touch base on their respective activities, discuss common issues and exchange on best practices. This fruitful meeting brought together over 38 members of the NMOs, EHC representatives and EAHAD CEO, Aislin Ryan, along with clinicians, invited speakers and industry representatives.



*Participants at the 2019 Nordic Meeting*

The workshop began on Friday afternoon with a very warm welcome from the Danish NMO Chairperson Jacob Anderson and staff member Karen Binger Holm, followed by a 10-minute update from each NMO about what is currently happening in their country, some of the successes they have experienced over the past year, the next focus for their NMO and some of the issues and challenges ahead. This was an opportunity for NMOs to exchange and share experiences, but also to ask lots of insightful questions about how things are organised and implemented in other NMOs. The EHC also provided the attendees with an update of what we are doing, when we are doing it and why we are doing it!

Saturday morning began with a conversational style session between Dr Peter Kampmann, Chief Physician, Rigshospitalet, and the Chairman of the Danish Haemophilia Society, Jacob Andersen. They discussed the **“Tarzan Syndrome”** whereby patients can sometimes refuse treatment, pain relief and try to avoid being identified as having a bleeding disorder which can lead to unnecessary pain and bleeding problems. The pair discussed the affectionately named **“Stiff-Ankle Society,”** a group for patients aged between 30 and 49 years. Dr Kampmann highlighted that both clinicians and patients can learn a lot from each other in this type of group, by discussing the challenges they face with pain, injuries and managing their identity alongside the management of their condition. This conversation was both honest and eye-opening from the perspective of re-adjusting our expectations as we age.

This was followed by a report on the Mind Study from Sweden, which gathered data from 1550 people from 2007-2017, exploring health-related quality of life, pain management and the incidence of depression and anxiety in patients with bleeding disorders conducted by the Swedish Institute for Health Economics. The data showed patients with haemophilia have increased levels of acute and chronic pain and long-term disability, and are at a higher risk of continued use of psychotropic drugs than the average population.

A main theme throughout the weekend regarded the need for Comprehensive Care Centres for patients with bleeding disorders, with many centres not yet certified as comprehensive haemophilia treatment centres in the Northern and Baltic countries. A panel discussion with Aislin Ryan, Dr Eva Funding, Rigshospitalet, Morten Skovhus Eriksen, patient representative, Denmark and Morten Frank Pedersen, Chair, Danish Association of the Pharmaceutical Industry Rare Disease Committee explored the future needs for comprehensive care. Each speaker agreed with the goal and the importance of ensuring that patients have access to a Comprehensive Care Centre, with Dr Eva Funding emphasising that the future of haemophilia care is becoming more complex with patients living to older age and the management of new and novel therapies. Dr Funding stressed that we must aim to ensure a high level of expertise in order to safely and effectively manage this new era of haemophilia and bleeding disorders treatment and care. Patient representative Morten Eriksen reinforced this mantra, by explaining that patients with bleeding disorders need a treatment home and expert resources when, and if, complex issues arise.

A parallel programme for young members from the represented NMOs was held from Saturday afternoon to engage and explore topics of interest with the already motivated and empowered youth. Consisting of 12 young people, the group took part in an interactive session on motivation and community building with Casper Rongsted, Educational Consultant & Writer, Rongsted Learning and Motivation, who worked with participants on maintaining their motivation, leadership and engagement in the community. On Sunday, Fiona Brennan facilitated an interactive action planning workshop on participants involvement, taking into consideration the current and future activities of the youth in the Nordic and Baltic NMOs. The future of these NMOs continues to shine bright with some inspirational, motivated and educated youth ready to engage actively with their NMOs and with each other!

The weekend ended with an interactive evaluation and discussion on current issues that the Nordic group agreed to work on together, namely developing a consensus statement regarding Comprehensive Care Centres in the Northern and Baltic countries.

*We send our warmest thanks to the Nordic Haemophilia Societies for the kind invitation and warm welcome! We were delighted to be able to share an insight into this year's meeting and look forward to seeing this engagement further empower patients and NMOs well into the future!*

## Nothing about us without us

Germany passes a new law that moves the distribution of haemophilia products from treatment centres to pharmacies

*A legislative proposal could have harmed haemophilia care in Germany, but thanks to the work of the national patient organisation and other stakeholders, the legislation was amended to address patients' concerns.*

At the end of last year, the German Ministry of Health proposed a new law affecting the distribution of haemophilia treatment products. So far, all products are distributed by the haemophilia centres directly to the patients. In future, patients will get them from regular pharmacies.

When the German Haemophilia Association, DHG, heard about the Ministry's plans last year, we were very concerned. We feared that the new law would destroy a well-established system and lead to numerous problems, such as:

- the erosion of haemophilia treatment centres,
- a decrease in quality of treatment, and
- a threat to treatment safety, traceability and documentation.

In our eyes, the draft was developed too quickly without considering all consequences.

So, we started our campaign. We also asked the EHC for help and received a great 'letter of support,' which was very useful for us. In February 2019, we had the opportunity to give a presentation about the proposed law at the EHC Round Table held in the European Parliament in Brussels under the auspices of Member of European Parliament (MEP) Mrs Gesine Meissner (Germany/ ALDE). After the Round Table, we decided together to send a request to all NMOs of the EHC. The goal was to get a better understanding of how the other European countries distribute factor concentrates. We got replies from many different countries which were very helpful.



*Dr Anna Griesheimer presented the situation in Germany during an EHC Round Table of Stakeholders*

At the same time, we were able to establish contacts with the relevant health politicians of all German political parties. Additionally, we asked our members to contact their respective Members of Parliament (MPs). To support them in doing so, we sent out sample letters. Soon we noticed that we managed to make haemophilia a topic of discussion in Berlin, Germany's federal legislative capital. During the readings of the proposed law in the German federal parliament, many different politicians addressed the topic of haemophilia.

In April, there was a public hearing, and the DHG was invited as an expert to give testimony. We were also asked to provide a written statement. In May, we organised a Round Table in Germany with doctors, representatives of health insurances and pharmacists to discuss concrete proposals and find adequate solutions. Simultaneously, the Ministry of Health, together with two MPs, organised an 'expert meeting' about haemophilia to which we were also invited. In summary, we were in a lively exchange with many politicians and had profound discussions with all relevant stakeholders during the legislative process.

In June, the law was passed by the parliament as well as by the Federal Council. As we had anticipated in the weeks prior, we were not able to prevent the change of the distribution channel from haemophilia centres to pharmacies. However, we are happy that our objections were taken seriously and that the draft law was amended to take some important details into account. The amendments that were incorporated in the final legislation strengthen the role of the haemophilia centres and oblige pharmacies to report to the prescribing doctors, which closes the potential gap in documentation which we had originally feared.

This new law, regarding the distribution channel of haemophilia products, will come into effect in August 2020. In the upcoming months, all stakeholders will prepare for the change. We will carefully monitor the process and look out for potential problems. But after all, we are confident that it will be possible to find workable solutions.

*We want to thank all of you who provided us with information about the distribution system in your respective countries. We appreciate it very much that so many of you were willing to help. Last but not least, we wish to thank the EHC team for their helpful support!*

## One more piece of news from our NMOs

### **EHC features NMO summer camps in video**

In September, the EHC launched a call to our NMOs to submit videos and photos from their summer camps. EHC staff compiled all the material submitted into one video to showcase its NMO summer activities. Each year a huge percentage of our NMOs hold summer camps for children and teenagers with a host of activities and educational sessions including self-infusion and physiotherapy! The EHC wishes to thank all NMOs who submitted their summer memories.

[Watch the four-minute video here.](#)

## Feature Articles

### THREE, TWO, ONE – LET IT RIP!

#### Patient education is taken to a whole new level!

Pegasus special move – Storm Bringer!!! If you are a parent of a 9-year old, you surely have gone through the phase of Beyblade! Or you may be familiar with One Piece or Dragon Ball if there are teenagers in the house!

Channelling Manga – the Japanese cultural phenomenon that has become so popular with the younger generation all over the world – into the haemophilia patient education, means bringing it to the whole new level!



**Dr Yasuharu Nishida** is a haematologist working for Osaka National Hospital in Japan. Together with his colleagues, he has developed a new educational tool about carriers of haemophilia – in the form of a Manga book **“Hitorijanaayo (You are not alone)”!**

Through the lens of a young girl who is a carrier of haemophilia, this Manga explains the basics of haemophilia, that is not always easy to understand for younger kids. Starting from the components of our blood and their functions, to the clotting cascade, haemophilia A and B and the severity of the condition. A section of the Manga is dedicated to how one inherits haemophilia or becomes a carrier, and how the clotting factor activity in the blood can be tested.

However, the main focus of the material is on what it means to be a young girl and a carrier of haemophilia from a physiological, emotional and psychological perspective, especially when it comes to dealing with your own newly-discovered diagnosis and your identity in a family where the main focus is on a boy who has haemophilia.

Here below is a short interview with Dr Nishida, about how this project was conceived and the impact it has had on the community.

#### **Dr Nishida, could you please tell us who conceived of this project?**

I am pleased to have this opportunity to share our new resource about issues young carriers of haemophilia face.

As you know, there is no denying that the care for carriers has remained unchanged in comparison to the amazing improvement of haemophilia care itself. In my practice, I have seen too many carriers suffering from an unspoken bleeding tendency, becoming isolated and finally being separated from the outer world. This harsh reality prompted me to work on raising the awareness of the issues carriers face in Japan, taking advantage of every opportunity for doing so, such as lectures, webinars and booklets. However, I found it rather difficult to convey messages to pre-teen carrier girls who are generally less likely to read complicated booklets.

At the same time, my colleague Dr Taemi Ogura, a pediatric haematologist in Shizuoka, was struggling with the same difficulty of educating pre-teen carriers and came up with the idea of using Manga.

#### **Why the choice of a Manga? How long did it take to create it? How was it developed and financed?**

For decades Manga has obtained a strong position in Japanese culture. Today, Manga is popular among all age groups in Japan, from young schoolgirls to ageing corporate executives, and span a remarkable

range of subjects, including action, romance, science fiction, food and history. It has been recognised as a popular method for sharing messages to people of all ages.

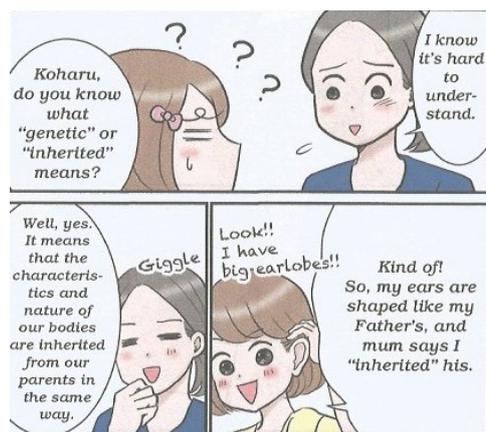
We were fortunately supported by Chugai Pharmaceutical (a member of the Roche group), who backed us up in the implementation of this idea, not only financially but also practically by looking for the best Manga artist (Hatoko) and a reliable publisher (Kadokawa). This way, we could deliver a highly relevant and also a high-quality educational resource.

The Manga book entitled “Hitorijanaayo (You are not alone)” was launched in June 2018 in Japan, after 11 months’ of preparation. Since then, it has been getting more and more popular.

### **What are your hopes for distributing this material in patient communities globally?**

The success of this material motivated me to share the Japanese Manga with such overseas experts in haemophilia as Dr Roshni Kulkarni in Michigan, Dr Peter A Kouides in Rochester, New York, Dr Robert Sidonio Jr in Atlanta and Dr Rezan Abdul-Kadir in London. I am very grateful to them because they encouraged me to translate it into many languages and distribute it to the patient communities with the help of EHC and WFH.

Following this encouragement, our Japanese team members devoted themselves to translating the Manga into English, to begin with. In March 2019, we completed the English version and were happy to share it with the EHC and WFH. I do hope that this will gain similar popularity overseas and help us all to make more progress in the care of carriers in Japan and globally.



### **What are the future projects that you are working on?**

As I told you, “You are not alone” has caught on among Japanese pre-teens. Following this success, we just launched a new Manga for young adult carriers that tells a story about a carrier who is struggling for a happy and fulfilling marriage. This takes the angle of a romantic comedy. I really do hope that this material will help the young adult carriers reduce the stigma surrounding them and enjoy their diverse lives!

Our heartfelt thanks and congratulations go to Dr Nishida and his colleagues for such an excellent idea and useful material that will surely help many young girls to understand what it means to be a carrier of haemophilia! Dr Nishida and his colleagues are more than happy to share this resource to be translated and used in your local contexts! Please contact us at the EHC if you wish to do so!

## Navigating through a transformed European landscape

### Taking stock: the European elections

*In May 2019 the citizens of the European Union cast their vote and chose a new European Parliament. With the highest turnout in the last twenty years - slightly over 50% - these elections featured a general rebuff of traditional mainstream parties. A much-feared move to the right did not take place, yet new leading political forces will create new majorities and momentum to re-evaluate the "European idea."*

The traditional "grand coalition" between the Christian-Democrats (EPP) and the socialists (S&D) was dismantled. During the last decades, these two political families were the most dominant political groups in Brussels. Today, although the EPP and the S&D Groups remain the two largest, they no longer enjoy total dominance. Only with the liberal Renew Europe Group (formerly known as ALDE) or the Greens, both the winners of the elections, can majorities be formed. Renew Europe became the third biggest political force in the European Parliament, while the Greens have advanced to the fourth place. This new balance will undoubtedly influence the upcoming European agenda. Overall, the political landscape in Brussels has become more fragmented and finding political compromises on the EU level will be challenging.

Following the European elections, the European Council surprisingly nominated on 2<sup>nd</sup> July the incumbent German Minister of Defence, Mrs Ursula von der Leyen, to become the next President of the European Commission. The European Parliament confirmed her on 16 July.

Besides a longstanding political career, Mrs von der Leyen is a medical doctor by training and profession, focused on women's health. Having someone with a medical background at the helm of the European Union brings renewed hope for advocacy groups, such as the EHC, that the European institutions will continue to promote and advance health-related policies and initiatives. Mrs von der Leyen has already started to build a work programme for the European Commission as well as a team of Commissioners to execute such a programme. She has called on the Member States to put forward their 'best candidates.' Mrs Stella Kyriakides, the Cypriot candidate, was appointed to lead the health portfolio. The appointed Commissioners will be interviewed by the European Parliament during the Commissioners' hearings to take place on 1<sup>st</sup> and 2<sup>nd</sup> October.

#### What to expect from the 2019-2024 mandate?



There will be much work to be done in the 2019-2024 mandate by the new Commission and Parliament on ongoing and new files related to healthcare. Amongst others, there will be a joint evaluation of the Orphan Medicinal Products (OMP) Regulation and the Paediatrics Regulation. This assessment is expected to be finalised in the fall of 2019. The Proposal for Health Technology Assessment (HTA) Regulation awaits approval in the Council of the EU. Aimed at ensuring consistent, high-quality HTAs, the new HTA

Regulation wants to improve access to new health technologies and avoid duplication of research. Negotiations are ongoing on Horizon Europe, the EU's framework programme to support research and innovation. It is also forecasted that in 2021, the European Commission will re-evaluate the functioning of the European Reference Networks (ERN), the EU's virtual networks of healthcare professionals spread across the Union to simplify discussion and knowledge-sharing on rare diseases and their treatments. This revision could entail opportunities for discussions on enhanced patient involvement in ERNs. Lastly, following years of assessment and evaluation, the Blood, Tissues and Cells Directive may also be re-opened for revision in this term.

Besides these legislative files, it is also expected that the Commission and Parliament will have to face other challenges such as access and affordability of medicines, medicines shortages and preparedness for Brexit.

A new political term brings fresh opportunities for working towards shaping future EU policies in the field of rare diseases. With a new balance of power and a new cohort of policymakers, it is a crucial time for building awareness around rare bleeding disorders.

#### **The MEP Rare Bleeding Disorders Group: where to go next?**

Since 2009, the EHC gathered committed supporters amongst Members of the European Parliament. In 2015, these supporters gathered in an informal group of MEPs supporting patients with rare bleeding disorders. The Group formalised in November 2017 to the MEP Rare Bleeding Disorders Group.

During the last two parliamentary terms, these MEPs helped the EHC in raising awareness about issues faced by the European bleeding disorders community through its Round Table of Stakeholders. These events are held three times a year and allow a variety of stakeholders involved in the provision of care for people with bleeding disorders to meet, analyse a topic and discuss solutions and a way forward. Topics discussed in the Round Tables included recently, for example, the future of comprehensive care, switching from standard therapies, and women and bleeding disorders. With so many innovative treatments for haemophilia under development, the topic of access and affordability of novel technologies has been frequently discussed during the Round Tables. The EHC is hoping to bring discussions on 'Markets and Access' again to the European Parliament on 3<sup>rd</sup> December for its next Round Table.

Although the European elections slimmed down the MEP Rare Bleeding Disorders Group to only one member, Dr Cristian-Silviu Buşoi (EPP, Romania), the EHC is confident that, thanks to its track record, it will be able to attract new MEPs to the cause of people with rare bleeding disorders in Europe. In particular, the EHC is looking for MEPs that will champion its activities including access to novel therapies, support for people with inhibitors, the development of young volunteers, recognition for women with bleeding disorders and the support of an ageing patient population, the first one of its kind.

The EHC is looking forward to renewing its collaboration with the European Parliament to advance the cause of people with rare bleeding disorders in Europe.

## News from our corporate partners

The EHC would like to acknowledge and thank the 2019 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
- Sobi

### Gold:

- Bayer
- CSL Behring
- Novo Nordisk
- Sanofi-Genzyme
- Takeda (previously Shire)
- Spark

### Silver:

- Biotest
- Roche



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



## 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 are proud to continue supporting the following patient / physician tools and educational activities in 2020



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.\*

The **Haemoassist® 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® 2** tool supports people with haemophilia regardless of their choice of clotting factor concentrate.†



Throughout 2020, Pfizer will continue to support the European Haemophilia Consortium (EHC) by contributing funding towards the following EHC-organised activities:‡



\*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.  
 †StatConsult is the legal manufacturer of Haemoassist® 2 and Pfizer holds sole distribution rights. Haemoassist® 2 is a CE-marked medical device in Europe, currently available in Germany and Spain, with planned additional launches in 2020. It is currently neither registered nor available in other regions. Haemoassist® 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® 2.  
 ‡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 service and tools, contact your local Pfizer team.

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 PP-HEM-GLB-0056  
 Date of preparation: January 2020

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



## 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 are proud to continue supporting the following patient / physician tools and educational activities in 2020



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.\*

With over 600 healthcare professionals trained over the last 7 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.†



Throughout 2020, Pfizer will continue to support the European Haemophilia Consortium (EHC) by contributing funding towards the following EHC-organised activities:†



\*These programmes have been initiated and funded by Pfizer.

†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 service and tools, contact your local Pfizer team.

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## unlocking a world of possibilities for people with haemophilia

Close collaboration with the haemophilia community remains an integral part of Sobi's work, which is why we were so proud to support EHC's priorities again in 2019. There were several highlights throughout the year, all of which have contributed to the strength of our ongoing partnership.

### Sobi's research revealed true unmet needs among people living with haemophilia

Ethnographic research presented by Sobi at the 2019 EHC Congress found that despite developments in approaches to treatment in recent years, people living with haemophilia (PwH) still carry burdens of the condition on a daily basis.

The study, conducted among 51 people living with haemophilia in several EU countries and 18 healthcare professionals revealed that there is still an unmet need for more flexible and personalised approaches to care to allow PwH to lead lives beyond the bleeding disorder.

The research was presented at the Sobi symposium during the 30th anniversary EHC Congress in Skopje.

"The research shows that most people with haemophilia still live a life far from normal. For people with haemophilia, a 'normal life' involves living with restrictions and regular burdens of the condition. This is a result of conservative approaches to treatment aiming to achieve basic protection rather than overcoming limitations and expanding possibilities. The study also found that some current treatment approaches may not even be sufficient to deliver basic protection," - Philip Wood, Head of Haemophilia Senior Vice President at Sobi.

### Liberate Life: a new vision of living life beyond haemophilia

The ethnographic research conducted by Sobi exposed many challenges, aspirations and unmet needs related to life with haemophilia. Inspired by these insights, Sobi then launched a new vision to coincide with World Haemophilia Day 2019 called Liberate Life.

Such vision symbolises Sobi's intentions and actions and embodies our dedication to working for people living with haemophilia, enabling them to be protected from bleeds, joint damage, pain and mental burdens – helping them feel safe so they can live the life they want, with more possibilities.

"Liberate Life not only spells out our vision for the future of haemophilia care but also serves as a call to action for involvement from patients, advocates and healthcare professionals." – Guido Oelkers, Sobi President and CEO

Liberate Life is a vision made effective through partnership with everyone living with haemophilia, their family members and friends, and the professionals who care for them. By encouraging and motivating the broader haemophilia community across the globe to expect more from life and to reach for the many possibilities that advances in care have made possible, Sobi believes that we can challenge the status quo of today's haemophilia care together.



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## 2019 Sobi Liberate Life Bike Ride

The 2019 Liberate Life Bike Ride was the fourth Sobi charity ride to raise funds for the European Haemophilia Consortium (EHC). A team of cyclists from Sobi rode over 520 km over three and a half days through Wuppertal, Bielefeld and Verden on the way from Bonn to Hamburg.

The cyclists were 13 Sobi employees from across Europe, a mix of dedicated semi-professionals and people who normally just bike to work. The Liberate Life Bike Ride is not a race, but rather a team event where all team members work together to ensure everyone reaches the final destination.

As well as raising funds for the EHC, the Liberate Life Bike Ride also aims to build awareness about haemophilia and the benefits of an active life for people with the condition. The Bike Ride is also an important opportunity to highlight the Liberate Life vision for people living with haemophilia.



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## Empowering the Hemophilia Community to Break Boundaries and Thrive



At Bayer, we're dedicated to helping people living with hemophilia achieve their full potential. Through our evolving patient support programs we're helping to redefine what it means to live with hemophilia.

### Be a Leader in the Community

#### HEMOPHILIA LEAD

A collaborative leadership development platform designed to help advocates with the power to become the voice of the community and address challenges and barriers to care. Its programs provide skill building and training for people with hemophilia around the world and across many stages of life and experience.



**WHO:** Men, aged 21-25, diagnosed with hemophilia A or B, or a related bleeding disorder  
**WHAT:** A one-year international leadership development program



**WHO:** Men or women, aged 26-38, with either a diagnosis of a bleeding disorder or working in hemophilia advocacy  
**WHAT:** A two-year international fellowship program to grow leadership and advocacy skills

Learn more at [www.hemophilialead.net](http://www.hemophilialead.net)

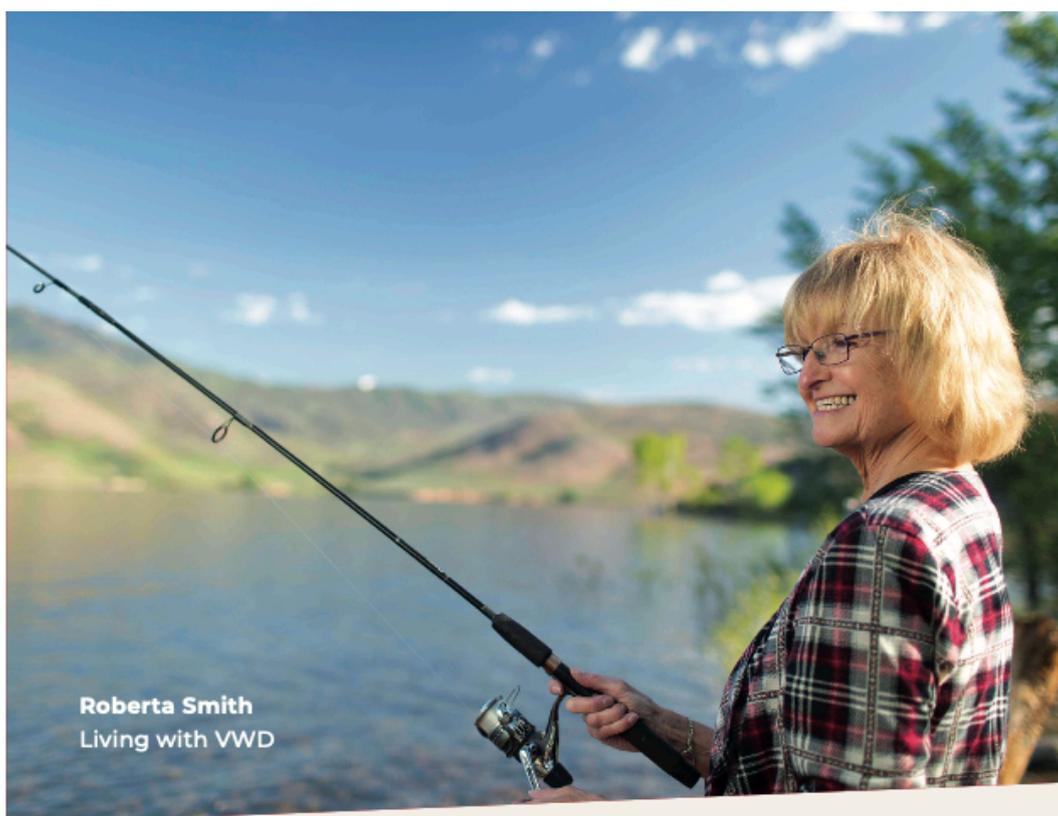
### Take Control



A patient-powered hemophilia service that allows you to track adherence, outcomes, well-being and mobility via an app. Visit your treatment center for more information.



A personal companion that empowers people with hemophilia to self-monitor FVIII level and stay informed of their prophylaxis regimen. More information is available at: <https://mywapps.org>. The myWAPPS app is free to download for iPhone and Android.



**Roberta Smith**  
Living with VWD

## 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**  
Biotherapies for Life™



Together we are  
driving change in  
haemophilia

Novo Nordisk is committed to driving change in haemophilia. Our key contribution is to discover and develop effective and safe medicines for people with haemophilia and other rare bleeding disorders. But we know that to truly change haemophilia, we need to do more than supply the right medicines. That is why we work with our global partners like European Haemophilia Consortium (EHC) to advocate for and create better access to diagnosis and multidisciplinary care. Changing Haemophilia® is our commitment to address the unmet needs in haemophilia.

Learn more at [novonordisk.com/changinghaemophilia](https://www.novonordisk.com/changinghaemophilia)

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changing  
haemophilia®

  
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## Congratulations to the EHC for their 30 years of successful advocacy!

The past year marked the European Haemophilia Consortium's 30-year anniversary. In these three decades, the EHC has significantly advanced the standard of care for people living with hemophilia in Europe through successful advocacy.

We are proud to have supported that journey through our legacy companies and now as Takeda. We will join the journey onward, driven by our belief that **every bleed matters**. Everyone is unique and every experience of a person with a rare bleeding disorder is different. All people with bleeding disorders, wherever they live, deserve a high standard of comprehensive, personalized care.

Takeda has been a supporter of many of the EHC's programs since their beginning until the present day, including the Roundtables, the Youth Leadership Workshop, the European Inhibitor Network and the Annual Conference. In 2019, a highlight was the publication of the EHC's Documentary on Women and Bleeding Disorders, which Takeda supported with an independent grant.

Takeda commends the EHC and its national member organizations for raising awareness of the high unmet need in rare bleeding disorders. We look forward to continuing to support this work, so that everyone with a rare bleeding disorder can live their life to the fullest.

[www.takeda.com](http://www.takeda.com)

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# Creating the path for advancements in hemophilia 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



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:

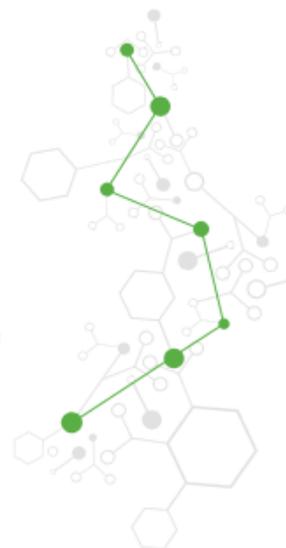
Contact [patients@sparktx.com](mailto:patients@sparktx.com) for more information.



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For more information, please visit [SparkTx.com](http://SparkTx.com)

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Being a

# Community Partner

for all who are touched by haemophilia



*Ensuring Treatment for All, by partnering with patient organisations to help make better treatments available to all people with haemophilia A who need medication.*



*Supporting the Person with local programmes that aim to improve health and well-being of people with haemophilia A, their families and caregivers.*



*Furthering the Science by reaching beyond the status quo to deliver medical innovations that matter.*

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## CALENDAR OF EVENTS

### EHC 2020 EVENTS

18 February	Round Table of Stakeholders on <b>“Haemophilia Registries and Clinical Outcomes”</b> <i>Brussels, Belgium</i>
April 3-5	<b>Youth Leadership Workshop</b> <i>Amsterdam, the Netherlands</i>
April 24	<b>World Haemophilia Day</b> <i>“Open the door to another reality”</i> <i>Brussels, Belgium</i>
May 18 or 19	Round Table of Stakeholders on <b>“Patient-Clinician Education Models”</b> <i>Brussels, Belgium</i>
May 29-31	Conference on <b>“Women and Bleeding Disorders”</b> <i>Basel, Switzerland</i>
June 25-28	<b>Leadership Conference</b> <i>Brussels, Belgium</i>
July 1-5	<b>EURO 2020:</b> Adapted Football & Physical Activity Camp <i>Utrecht, The Netherlands</i>
September 11-13	Workshop on <b>Tenders and Procurement</b> <i>Helsinki, Finland</i>
October 2-4	<b>2020 EHC Conference</b> <i>Copenhagen, Denmark</i>
October 26 or 27	Round Table of stakeholders on <b>“Gene Therapy: Economics, Affordability and Exclusions”</b> <i>Brussels, Belgium</i>
November 20-22	Workshop on <b>New Technologies in Haemophilia Care</b> <i>Location TBC</i>
December 3-6	<b>Inhibitor Summit</b> <i>Barretstown, Ireland</i>

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