Haemophilia.ie

Magazine of the Irish Haemophilia Society

Representing People in Ireland with Haemophilia, von Willebrands & Related Bleeding Disorders

SCAN ME

Summer 2024 Edition

From the Editor

Hello everyone! I hope you are all set for summer and that you have the opportunity to enjoy some sun over the next few months, be that in Ireland or abroad!

We have a jam-packed edition for you this quarterly magazine. As is customary, Brian O'Mahony will open the magazine with his CEO's report, and I would recommend members read this as it includes some problematic updates on World Health Organisation (WHO) guidelines. After this, John O'Mahony gives a summary of the most interesting points raised at our Ageing Conference just gone in May.

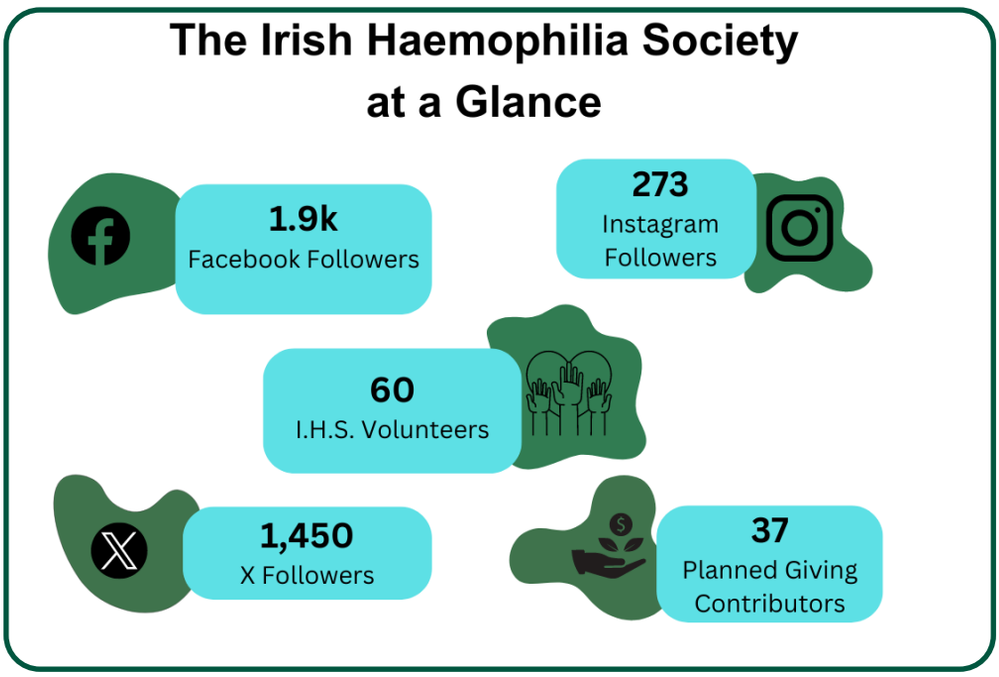
Following this is my own highlight of this edition - the interview I conducted with Dr. Assad Haffar, Medical and Humanitarian Aid Director for the World Federation of Hemophilia. In this interview, Dr. Haffar relates key developments in his career at WFH over the past 24 years, as well as developments in the aid programme WFH created in order to help countries that do not have adequate treatment. Dr. Haffar gave a brilliant talk on a similar subject at our AGM this year and it was an honour to get to discuss his work with him privately for this interview and ask him any questions I had.

In April, Brian O'Mahony, Debbie Greene, Nina Storey and I, along with John Stack and Dan McIntrye from the Board, headed off to Madrid, Spain for the WFH Congress. I went early as I took part in the national member organisation (NMO) training beforehand. This was a fantastic experience for me and I record my key highlights from this later in the edition. Following this, the rest of the IHS staff and the board who attended provide their highlights from the sessions they attended at the Congress.

In January of this year, Con Walsh and his family led a tractor run in Co. Cork and raised €1,900 for the Society! His daughter, Michelle McKeon, has written a piece for us on this wonderful achievement.

Last but not least, I urge members to take a look at the closing pages of the magazine as there is information on our upcoming strategic plan for 2025-2028 and details about our Von Willebrand & Rare Bleeding Disorders Information Day taking place in July.

Roisin Burbridge, Publications, Website & Social Media Coordinator



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CEO Report

Ageing Conference

The Society Ageing Conference took place in Tralee in May. The conference was very well attended by approximately 40 members. Speakers included Professor Mike Makris from Sheffield, Ms. Patricia Byrne from the National Coagulation Centre (NCC), Dr. Cleona Duggan from Cork University Hospital and Mr. David Page from the Canadian Hemophilia Society. It was wonderful to see the warm sense of community which prevailed at the weekend with participants sitting outdoors in the sunshine at coffee breaks and re-connecting with old friends and making new friends.

Centre audits

Peer-reviewed audits of the comprehensive care centres at St. James's Hospital, CHI Crumlin and Cork University Hospital took place in late May with a team of auditors from abroad. The team comprised an adult haematologist, a paediatric haematologist, a physiotherapist, a nurse, a psychologist, and a patient representative. The patient representative, Mr. David Page from the Canadian Haemophilia Society, has assisted us on several previous occasions. These audits play a crucial role in assessing and enhancing comprehensive care in Ireland. Having a team of international experts scrutinise the provision of services, treatments, and care for individuals with inheritable bleeding disorders is invaluable. The auditors examine every facet of comprehensive care, asking questions as to why things are done or not done and offering recommendations, which are listened to carefully by the hospital chief executives. A significant aspect of the process involves anonymised patient surveys conducted beforehand to gauge patient perspectives. The surveys, distributed by the Society via SurveyMonkey, garnered approximately 30 responses. Additionally, separate surveys were sent out by the comprehensive care centres using a different SurveyMonkey platform. All survey results were sent directly to Mr. David Page, the patient auditor, who compiled a report based on these findings. The anonymity of survey respondents is strictly maintained, ensuring the confidentiality of their feedback. This feedback provides crucial insights into the audit process. The audit reports are not only shared internally but are also submitted to the National Haemophilia Council for further review of their implications for comprehensive care.

Retrograde steps for haemophilia care internationally

A recent development has shone a light on a potential threat to access to haemophilia care. This was the decision of the World Health Organisation (WHO) to place cryoprecipitate on the Essential Medicines List (EML). The WHO produces an updated EML every second year. This includes a core list of the most efficacious, safe and cost-effective medicines for priority conditions and a complementary list which presents essential medicines for priority diseases for which specialised diagnostic and monitoring facilities and/or specialist medical care or training are required. The core list include the most important medicines and signal to countries that these medicines should be widely available.

In 2003, at a time when I was President of the World Federation of Hemophilia (WFH) the WHO recommended that factor VIII and factor IX plasma-derived clotting factor concentrates be considered

for removal from the EML. Following extensive submissions and work from the WFH, the WHO expert committee rejected this recommendation and retained plasma-derived factor concentrates on the complementary medicines list. This was very welcome at that time. The WHO committee noted that *"factor VIII and factor IX concentrate are life-saving in the treatment of haemophilia and the alternative (cryoprecipitate from whole blood donations) is less safe and more expensive."* Cryoprecipitate is a product made from plasma usually recovered from whole blood donations. It is not virally inactivated and therefore is still capable of transmitting blood borne viruses, including HIV or hepatitis viruses. In more recent years a pathogen-reduced version of cryoprecipitate, which protects from some but not all viruses, has been developed and used in a very small number of countries. Cryoprecipitate is not a product which should be used to treat haemophilia unless there is absolutely no alternative. It is not a safe product. Pathogen-reduced cryoprecipitate is safer but still not totally safe and certainly does not have the safety profile of plasma-derived factor concentrates.

Inexplicably, the WHO, in their 2023 revision of the EML, placed pathogen-reduced cryoprecipitate on the core EML, with non-pathogen reduced cryoprecipitate listed as an alternative. Simultaneously, they left plasma-derived coagulation factors VIII and factor IX on the complementary EML. This places unsafe products above safe and effective plasma-derived factor concentrates. Despite strong representations from WFH since then, WHO have refused the request to remove cryoprecipitates from the EML and to relegate pathogen-reduced cryoprecipitate to the complementary list and place safe and effective plasma-derived factor concentrates on the core list. This inaction by WHO could lead to these unsafe products being used, especially in low or medium income countries, by governments who do not wish to purchase factor concentrates which cannot be made from locally sourced blood transfusions. WFH are continuing their efforts to have this decision reversed and to highlight the danger that this policy decision by WHO means for the community. This could cause morbidity or mortality and it could potentially give governments an excuse to use inexpensive locally produced unsafe cryoprecipitate as their factor VIII haemophilia therapy. This puzzling and erroneous position is due partly to the fact that the recommendations expert panel for WHO did not include any clinicians who treat haemophilia. It is also ludicrous in 2024 that recombinant factor concentrates, which have been on the market since 1994, extended half-life recombinant factor concentrates, which have been on the market since 2014, and subcutaneous mimetics on the market since 2018, are not even considered by the EML. The WFH continue their efforts to change this decision and to highlight the failure of WHO to promulgate appropriate recommendations for haemophilia treatment in their EML list. Clearly, WHO have forgotten or are ignoring the devastation caused to this community in the past by unsafe blood products. Those who do not understand history are condemned to repeat it.

In late May, I was part of a WFH delegation which met with the relevant officials at WHO during the World Health Assembly. We clearly outlined our grave concerns and we had a broadly constructive meeting. I do not believe that WHO will act expeditiously on this but I am hopeful that, in the revised EML in 2025, the WHO will heed our concerns and remove untreated cryoprecipitate totally from the EML, while placing PR cryoprecipitate on the complementary list as an alternate if not removing it entirely, which would be preferable.

Brian O'Mahony, Chief Executive

11. BLOOD PRODUCTS OF HUMAN ORIGIN AND PLASMA SUBSTITUTES	
11.1 Blood and blood components	
In accordance with the World Health Assembly resolution WHA63.12, WHO recognizes that achieving self-sufficiency, unless special circumstances preclude it, in the supply of safe blood components based on voluntary, non-remunerated blood donation, and the security of that supply are important national goals to prevent blood shortages and meet the transfusion requirements of the patient population. All preparations should comply with the WHO requirements.	
☐ cryoprecipitate, pathogen-reduced Therapeutic alternatives: - cryoprecipitate (not pathogen-reduced)	**Injection**: frozen liquid in bag or lyophilized powder in vial containing: - > 50 IU Factor VIII - > 100 IU vWF - > 140 mg clottable fibrinogen per unit
fresh-frozen plasma	

Review of the 202

At the turn of the century, the average life expectancy for people with severe haemophilia in Sweden was just 11 years of age but, today, that has risen to 77 and up to 84 for those with mild haemophilia. That was the reassuring message that keynote speaker Professor Mike Makris, Director of the Sheffield Haemophilia and Thrombosis Centre, conveyed to those in attendance at the Irish Haemophilia Society's Ageing Conference in the Meadowlands Hotel, in Tralee, Co Kerry. He said with modern treatments the 2024 target for people with severe haemophilia is zero bleeds and while that goal is somewhat aspirational, it is nonetheless very achievable. Outlining developments in haemophilia care and the advantages of prophylaxis, he pointed to the achievements of Alex Dowsett, a haemophiliac who has competed in the Tour de Franc, Chris Bombardier who didn't allow his factor deficiency to prevent him from climbing Everest and a team of eight haemophiliacs who have completed the New York Marathon.

Highlighting the real difference advances in treatment options have made, Prof. Makris also referred to one of his own patients with haemophilia who passed away just weeks shy of his 100th birthday. He didn't start treating prophylactically until he was 92, had hernia repair surgery at the age of 92 and underwent a total knee replacement well into his 90s.

"Life expectancy is increasing and now the older you are the higher the age that you consider old," he said.

Current treatment options include standard half-life and extended half-life products, bi-specific antibody treatments such as Emicizumab and Hemlibra, non-factor rebalancing therapies and gene therapy. Prof. Markris said he has some issues with the rebalancing agents and he stressed that there are still questions to be answered about gene therapy, such as how long it will last and if there are any long-term problems.

"There are many safe and effective treatments for haemophilia and many new therapies are in development," he said.

Prof. Makris said deciding which treatment to choose can be difficult but newer and more expensive options are not necessarily better and he advised those in attendance to seek multiple source advice on products before changing.

David Page of the Canadian Haemophilia Society told the two-day conference that in his country there is one known person with severe haemophilia over the age of 85 and 14 per cent of patients are 65 or older. A total of 24 treatment centres were set up in Canada between 1978 and 1982 with two more added since then and while some of the bigger facilities have over 1,500 registered patients, smaller centres cater for 100 or less. 65 per cent of Canadians are very satisfied with the service provided and distances to the nearest centre varied from less than 30km for 33 per cent of patients, and more than 300km for 16 per cent. David hosted

024 Ageing Conference

a very informative panel discussion involving IHS board members Breda Quealy and Dan McIntyre and Brian O'Mahony who discussed the need for resilience, their methods of coping with problems as they arose and their life experiences. The message from all three was quite simple – Don't sweat the small stuff.

On day one of the two-day conference, Dr. Cleona Duggan of Cork University Hospital said the national programme for the care of older persons is based on the need to standardise the quality of the service to accommodate their desire to live in the community for as long as possible and to promote independent living. She said the key elements of a comprehensive care centre for haemophilia should include haematology and hepatology, with access to physiotherapy and orthopaedic services and, where possible, psychiatric supports. Dr. Duggan said in terms of ageing, life expectancy for people with haemophilia 100 years ago was 11 for people with a severe condition and 28 for those classified as moderate. It has come a long way since then. The most recent medical research shows that people with haemophilia can now expect to live as long as those without the condition. Other age-related issues need to be considered, however, and challenges may emerge in relation to cardiovascular disease, renal problems, bone health, cancer and psychosocial issues. Early intervention and managing the risk factors was essential, the CUH Centre Director stressed.

Dr. Duggan stressed the importance of maintaining adequate levels of Vitamin D as a recent survey revealed that 87 per cent of the population had an unacceptably low level with a separate survey of national school children also giving cause for concern. Although she described the often stated goal of taking 10,000 steps a day as "a random figure plucked from nowhere without medical evidence", Dr. Duggan said that any movement is good and people should strive to keep as active as possible.

"Get up and switch channels rather than using the zapper," she advised.

In an engaging session at the conference, Patricia Byrne, Principal Clinical Psychologist at St James's Hospital, said the domains of influence in healthy ageing were exercise, sleep, mood and mindset, social connectedness, cognitive stimulation, nutrition and pain management. "It's never too late to build resilience," she stressed. She said in terms of pain management, options included pharmacological, surgical and physiotherapy but the mind also matters and there is a need to calm the nervous system and find ways to build distress tolerance and it often helps to embrace nature and the seasons.

In terms of mood and mindset, Ms. Byrne – who has a specific remit to provide evidence based psychological care to patients with haemophilia – said people should "have a go" in life and take calculated risks because, very often, change is good. She said people are living longer all over the world and some areas, in particular, are doing really well in terms of longevity, including Sardinia, Greece, Loma Linda in California and Okinawa in Japan and much of that can be attributed to diet and healthy living choices.

John O'Mahony, IHS Member

Interview with Dr. Assad Haffar

*Dr. Assad Haffar is the **Medical & Humanitarian Aid Director at the World Federation of Hemophilia**, where he has worked since the year 2000. Dr. Haffar is also a medical doctor with a specialisation in Public Health and Occupational Medicine.*

The below is an interview Roisin Burbridge carried out with Dr. Haffar at the AGM this year.

How long have you worked for WFH?
I started with WFH in the year 2000, so 24 years ago.

Could you tell me about the role you held as Program Officer for the Middle East and Africa?
At the time that I joined WFH, they were working to recruit programme officers to develop haemophilia care in certain countries. When I joined, they only had one programme officer, for the region of Latin America. They also wanted someone for the Middle East and Africa and that's the role I took on.

The WFH had very limited existence in these two regions. The year I started, only five African countries were members and only six or seven were members from the Middle East. So my task was to develop haemophilia societies in the different countries. I was also tasked with developing haemophilia care in these countries, identifying patients to advocate to government and delivering WFH assistance to the patients, doctors and ministries of health in these countries.

What was haemophilia care like in these countries before the WFH stepped in?
Most of these countries didn't have any haemophilia care. As you know, haemophilia care is very limited in countries with limited resources and some countries actually believe that it doesn't exist. I remember when we started working with Nigeria, the President of the Nigerian Haemophilia Society, who is a mother of two patients with haemophilia, went to the health authorities to advocate for haemophilia care and the director of health, who is a physician himself, told her that they do not have haemophilia in Africa. He claimed that it is a white man's disease.

Some countries did have knowledge because they had started haemophilia care a long time ago, like Egypt and Algeria. But most of the countries in the Middle East did not have any knowledge about it, so we were really starting from scratch in these regions.

What about countries like Saudi Arabia, Qatar etc?
Qatar did not know about haemophilia, while Saudi Arabia did have knowledge about haemophilia but not in an organised way. They had some resources but they needed a lot of help in order to know how to prioritise their goals. So when I started working with Saudi Arabia, we focused on the organisation of care.

Did you work directly with the hospitals or with the governments?
In Saudi Arabia, for example, we worked with the leading hospital in the country and this led to more communication with the government, getting to know patients and pushing for the identification of more patients.

I'm interested to know what care is like in that region now?
In the Gulf countries, care is great now. The work of the WFH has made a huge difference. Things begin to move much faster when we start working in a country.

Tell me about the humanitarian aid programme and what your role entails as the Humanitarian Aid Director?
The work of the WFH started to evolve more and more, particularly with more countries coming into the Federation as members. As more patients were being diagnosed in these countries, there was no treatment for them and there was no way to receive

treatment with the current resources these countries had. So the WFH needed to step in and provide some treatment. They did so through a new humanitarian aid programme. Over the years we have reached 112 countries through our donations. Today we reach about 80 countries.

Many countries that we helped in the past have reached some kind of self-sustainability, so that they don't need us anymore. Russia is an example of such a country. In the year 2001 and 2002 we used to donate to Russia. Now they have a good level of care and don't need our help anymore. This is the same with many countries in Eastern Europe, like Bulgaria and Hungary.

My role was to develop a good humanitarian aid programme, to ensure that countries complied with the regulations that have been set by WFH and the treatment donors. I oversee the humanitarian aid department to make sure that the treatment product is well received and transported in the country, stored properly, well used, in accordance with the WFH treatment guidelines, as well as traced to the end user, the patient, in case a product is recalled. I also ensure that the countries report back to us in the correct manner.

I want to divulge and talk about the role of the Irish Haemophilia Society (IHS) in the earlier years of the programme. During the course of the humanitarian aid donations, we really needed extra help to store, repackage and ship the products that we received to different countries. This is before we recruited the big distribution facilities that we have now, in Brussels, Memphis and Mumbai. This help came from two haemophilia societies. One was Haemophilia of Georgia in Atlanta, USA. The other was the IHS.

The staff of the IHS organised a kind of storage and distribution centre in Dublin to help us carry on our mission. For this, the IHS bought three fridges which they installed in their offices to store the product. I remember that at one point of time Declan Noone and Debbie Greene used to receive the product, unpack it, take the vials and place them in the fridge. Then when we sent them the donation papers they would repack the vials according to the distribution instructions we gave them. The product would then be shipped to the recipient country. This was a tremendous help and it came at a point of time when we couldn't receive the product in our headquarters in Montreal. This did put a lot of pressure on the IHS. We cannot thank the IHS enough, as they helped us at a really delicate time when we couldn't have continued our mission without them.

That's great to hear! What products are used by the humanitarian aid programme?
We started with the clotting factor concentrates, factor VIII and factor IX and the bypassing agents. Now when Emicizumab came to the market, we had communication with the manufacturer and we managed to get a donation from them for five years to treat 1,000 patients. We later renewed this for an additional five years and we managed to increase the number of patients on Emicizumab to 1,200.

That's great. How many countries does that cover?
We have 34 countries receiving Emicizumab.

And what about treatment for haemophilia B?
Unfortunately for haemophilia B, the product on the market is available less and less frequently. That's why we send less quantities of factor IX and why we cannot give patients with factor IX prophylactic treatment. We have to limit its use for acute bleeds. As well as the fact that less is on the market, we don't have donations from all manufacturers and two manufacturers do not give us enough factor for our programme.

Does the programme help other bleeding disorders like von Willebrand disorder (VWD)?
We have started receiving some products that treat VWD. This is really in the early stages, but we have started helping some patients with VWD. We have 500-600 patients with VWD being treated in our recipient countries.

Could you discuss the major milestones of the programme?

We have had many major milestones. The programme began in the year 1996, but when we started it was with very limited quantities of treatment with very short shelf life. We didn't know when the product would come to us. This is because whenever a manufacturer would fail to sell their treatment, they would donate it to the WFH. If we had known when the product was coming we would have set some kind of treatment protocols with recipient countries.

In 2015, we started the expansion of the programme, where we signed multi-year agreements with some manufacturers. This enabled us to set treatment programmes with recipient countries. So if the country had people waiting for surgeries we would tell them when the product was coming so that they could plan these surgeries accordingly. We also had products with a longer shelf life. We were now able to put people on prophylaxis with the increased quantities and longer shelf life of products. We could now advocate to governments and show them the value of treating patients with factor concentrates, rather than cryo or plasma. We could show them that treating patients with the right products would more quickly stop the bleeding.

Since the year 2015 when we started the expansion, 28,000 patients have been treated with our products. We now have between 12,000 to 14,000 patients who are being treated with donated products every year. This is a big milestone.

Another big milestone is the number of people on prophylaxis. We have reached approximately 6,000 patients with prophylaxis, either with factor concentrates or with non-factor replacement therapies like Emicizumab. The good thing is that out of this number, 75% are under the age of 10. Our aim is to prevent joint deformity or disease from developing in these children's futures.

The fourth milestone is the number of acute bleeds that we have treated. Since the start of the expansion in 2015, we have managed to treat 350,000 acute bleeding episodes.

In addition, we managed to conduct more than 4,500 surgeries in the countries that are receiving product from us. Out of these 4,500 which are both major and minor surgeries, there were about 1,100 cases that were life threatening or limb threatening. These cases included surgeries for inter-cranial or inter-abdominal bleeds or big thigh haematomas that were threatening the limb itself.

Are there any particular stories you would like to share to illustrate the change that the programme has made to people's lives?

I have hundreds of stories but I will just tell one or two. We try to give to countries where there are other ongoing issues such as civil war or other humanitarian aid crises. I remember the case of one patient from Yemen who had inhibitors and who had a big abdominal haematoma. There was no way he would be operated on in Yemen. The person managed to escape as a refugee to India and from there he contacted us and we managed to donate a bypassing agent to the centre in India so that the surgery could be carried out. This was a success and the person survived. What is nice about this story is that when he returned to Yemen he continued to communicate with us. He sent me a photo of himself living happily in the mountains there and later he sent another one showing he had returned to college. He got a diploma in finance and recently sent another photo showing he had got married and had his first child. We still have a relationship with him. This is what we aim for. We follow up with patients like this man to make sure they are doing well.

I remember another case of a patient from Kenya called Warren who I met first in 2013. He was about 10 years old and had chronic synovitis in his knee and needed a synovectomy. We sent the product to the hospital so they could carry out the procedure and this was a success. The boy had some physiotherapy after the procedure and when I saw him again in 2016 he was doing reasonably well with almost normal range of motion in his knee. I saw him again in 2018 and the last time I saw him was in 2022. It was great because he had gone back to school, has a girlfriend now and is thinking of getting married. What happened in 2013 has evolved so well over the past 10 years. Whenever I go back to the hospital in Nairobi I always ask to see Warren to see how he's doing.

What is the future of the humanitarian aid programme?

As I mentioned, we are receiving less products for factor IX and for VWD, so we are really trying to change this. We are in discussions with the manufacturers to see if we can either get more factor IX products or products that contain a high concentration of VWD. We also need more bypassing agents to treat patients with inhibitors.

At the same time, we hope to receive more of the newer products, as there are many rebalancing agents and bypassing agents in the pipeline. We are also involved in a clinical trial of haemophilia B gene therapy with St Jude Hospital in the USA.

Simultaneously, we are also trying to get donations of gene therapy from other manufacturers. We are hoping that in the future we will have two or three patients from one country who will receive gene therapy.

Gene therapy would be more cost-effective for governments.
Of course, and cost effectivity is very important for governments. Humanitarian aid can also be a very good advocacy tool. Like I said before, we show governments the value of treating with particular products. Once they see the result, they will invest more in haemophilia care. This is the same for non-replacement and rebalancing agents. We have examples of some countries that have started to provide Emicizumab or non-factor replacement therapies. We hope that when there are more non-replacement therapies and rebalancing agents on the market we will be able to convince governments to provide these to their patients. We managed to show the countries around Egypt that non-factor replacement therapies can be very good for patients. Now the Egyptian government has invested in the product and have almost 1,000 patients on Emicizumab. Other countries have also started investing limited funds to help some of the patients we could not reach with our programme.

What is life like for people with haemophilia in middle and lower income countries?
It is very difficult for patients with haemophilia in developing countries without humanitarian aid because, without us, these people have nothing at all. The president of one society in Africa said that having humanitarian aid from us is the difference between life and death. If they don't have it, the patient will die. Without help from WFH, patients die prematurely or develop premature joint disease. It is very hard for these countries to cope with haemophilia patients. When WFH step in and enable patients to receive treatment, this provides short-term positive results. But when health authorities step in and take over the purchasing of treatment this improves the sustainability of the haemophilia care in the country. We start with humanitarian aid and we end with the health authorities helping the patients, like in Russia and other parts of Eastern Europe.

How do you manage sending donations to countries who may be at war?
Yes, even if we can't go to the country, we can send donations. Like for example Yemen, we cannot go there but we have identified a doctor in a hospital in the capital who has agreed to receive the product and store it in there to give to patients. I know some people come to him from remote areas for treatment.

Have you been able to send donations to Gaza?
We are trying hard to help the patients in Gaza. We have tried so many avenues to send donations but none have materialized. We have assigned good quantities of factor to go to Gaza and they are in our distribution centre now. We are just trying to find the right avenue to send them. We have tried the WHO, and other organisations like the International Red Cross and Direct Relief, but we still need to keep trying.

Many thanks to Dr. Haffar for taking part in this fascinating interview.

WFH GNMO Training &

The World Federation of Hemophilia (WFH) Global National Member Organisation (NMO) training I attended prior to the Congress in Madrid was one of my favourite experiences since joining the IHS in 2022. I found it both highly educational and surprisingly emotional. The speakers, and particularly the workshop leaders, were really inspirational. Dawn Rottelini, Chief Operations Officer of the National Bleeding Disorders Foundation in the US, Deniece Chevannes of Hemophilia of Georgia, and Irene Bitharas of the WFH, brought a highly effective American energy to the workshops, full of passion and encouragement. While I would consider myself less than a little interested in finance, I found myself lapping up even Irene's finance workshop and found the budgeting activity fun and engaging. What made the workshops even better was the teams we were placed in to carry out the activities. Grouped with people from around the world, from Fiji to Trinidad to Myanmar, I had the opportunity to learn about haemophilia care in many different locations. Some of the stories were harrowing. I learnt, for example, that Fiji does not have a single haematologist in the country, and that the Society there have the responsibility of bringing factor to haemophilia patients when they have a bleed, often travelling for hours across the country to deliver the treatment. I saw first hand the effects of not having adequate prophylactic treatment, meeting several people under 40 with severe joint damage that has greatly reduced their mobility and ability to walk even short distances.

The plenary sessions further drove home the disparities in care across the world, with representatives of many countries sharing their struggles to engage government or the community at large regarding the needs of people with bleeding disorders. It became clear to me that we are in the minority in Ireland and that while conferences like WFH and our own in Ireland speak of revolutionary treatment like gene therapy, the majority of people in the world struggle to receive even basic care.

The training was also a pivotal moment for me personally, as I gave the first ever in-person presentation of my working life. The topic I spoke on was our VWD campaigns over the period of 2021-2023, focusing on our information days and information campaigns. Standing in front of WFH President, CEO and many other important faces, I thankfully managed to calm my nerves before starting my presentation. The countless times I had run over my slides enabled me to continue without many problems (apart from a few with the clicker!). I was most grateful that the presentation was at the start of the training and not the end!

Following the NMO Sharing of Experiences session, of which I was a part, there was a very interesting panel discussion with the head of the Society in Kosovo, Elmedina Kukaj and a few others who also shared their stories about working with various stakeholders to achieve their goals. I found it very moving to listen to Elmedina speak about the work she had done setting up a haemophilia organisation in Kosovo and building it up from nothing, aided by a supportive team and twinning partners in the USA. I could tell how important and personal her work was to her. She didn't stop beaming throughout the session and the pride she felt in her work was made evident through this smile. It was heart-warming to see.

After the plenary sessions, we started into the workshops. These really were the highlight of the training for me. I loved how interactive they were, striking a perfect balance between the facilitators sharing information with us and us having time to work on activities together and report back to the room at large. I had to report back on several occasions, as I was often the only native English speaker on my team! The topics we covered in the workshops were Good Governance, Working in Collaboration with Other Stakeholders, Project Proposal and Project Budgeting and Financial Reporting. The project my team worked on in the last few workshops was a plan for a rather ambitious camp for children to teach them how to self-infuse, while also providing pool diving lessons with proper scuba diving equipment! While the plan itself was too extravagant and potentially dangerous to take place in the real world, it was interesting to think methodically about all the stakeholders that would need to be engaged to make it work, including those who may be opposed to the project.

Over the course of the training, I learnt a lot of new information I will carry into my own work at the IHS, but more importantly, I made friends from all over the world who I hope to keep into the future.

Roisin Burbridge, Publications, Website & Social Media Coordinator

& World Congress Reports

Brian O'Mahony's highlights from the Congress

Madrid, April 2024

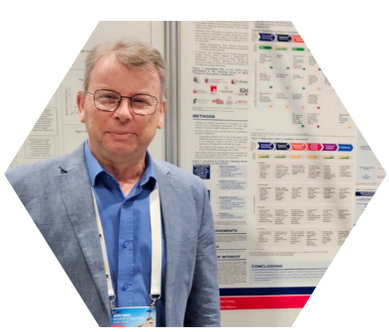
The WFH Congress, which takes place every second year, took place in Madrid in April this year. I was present at the WFH Congress the last time it was held in that city in 1988 and it was sobering to see how few people had been present that year. At one of the symposia I was speaking at, I asked the audience if anybody in the room had been at the Congress in 1988 and nobody had!

The Congress was attended by about 2,600 delegates, which was well below the figures of approximately 5,000 who attended congresses pre-Covid but double the number who attended the last Congress in 2022 when we were emerging from Covid. The Society was well represented by staff and board members and we each covered different parts of the programme. I delivered four talks during the course of the Congress and presented three posters.

My talks were on gene therapy, real world access and economics. They also included treatment options for low and middle income countries, making treatment decisions in a rapidly changing environment and using data in advocacy. My posters were based on the development of a gene therapy information clearing-house which brings together information and education materials from patient organisations and other relevant sources in the one place. They also featured results from a haemophilia B gene therapy meeting and the economic outcomes of a study of haemophilia in five large European countries. The gene therapy talk was based on a survey I carried out among doctors and haemophilia societies in 21 countries where I sought to ascertain the current stage of access to gene therapy in relation to health technology assessments in their countries, the ability to prescribe, as well as information on payment models and pricing. Not surprisingly, information on payment models and pricing was not readily available from many countries as discussions have not commenced, are at an early stage or are ongoing in many countries. Many countries will look at an outcome based or annual payment model in place of a one off payment model and countries may have to develop mechanisms to allow for this. I was very gratified to receive replies from all 21 countries; This 100% response rate is quite unusual. The licensed factor VIII gene therapy, Roctavian, can now be prescribed in the USA, Germany and Italy and the first licensed factor IX gene therapy, Hemgenix, can now be prescribed in the USA, France and Austria.

Some of the learnings for me from the conference in relation to the novel therapies in clinical trials included:

- Annual bleed rate was higher in non-inhibitor patients than in inhibitor patients with the rebalancing agents fitusiran and the anti-TFPIs.
- Fitusiran showed promise in mouse models for the treatment of factor X deficiency. This is welcome as there is currently only one factor X concentrate on the market.
- Concern was expressed by one speaker that, since people with haemophilia with existing thrombosis risk had been excluded from clinical trials with rebalancing agents, we may see an increased risk of thrombosis when these are licensed and more widely available. This points to the need to ensure this is taken into account by clinicians prior to prescription.
- One of the anti-TFPIs, marstacimab, showed a 35% decrease in annual bleeding rate when compared with previous prophylaxis.
- The new super extended half-life factor VIII, licensed in the USA as Altuviiio, extends the factor VIII half life by 3-4 fold. The standard dose in the clinical trials was 50IU/kg once weekly which, if infused intravenously twice per week, maintains a trough of at least 40% factor VIII for the first four days and no lower than 10-15% after a week. If this was used twice per week, possibly at a different dose, it could maintain the person with factor VIII deficiency in the normal range all week. This may turn out to be an option for those who are very physically active or involved in sports. This product is expected to be licensed by the European Medicines Agency (EMA) later this year.
- KB-V13A12, a novel nanobody based therapy being developed for von Willebrand



disorder shows real promise. In trials, it maintains the von Willebrand factor level at 11% for approximately 10 days.

- There was an optimistic discussion in relation to possibly re-purposing some of the rebalancing agents being developed for haemophilia A and B, with and without inhibitors, to also be capable of use in some of the rare bleeding disorders where current treatment options are very limited.
- Beqvez, a factor IX gene therapy just licensed in Canada and the USA, showed an average factor IX expression after two years of 25%. Of the 45 patients in the phase three clinical trial, six had returned to prophylaxis and 62% required steroids to deal with liver inflammation.

In relation to already licensed therapies:

- There was more information from clinical trials in small groups of people with factor VIII deficiency on the use of lower doses of Emicizumab (Hemlibra). A centre in Mumbai, India, used half doses and the results were similar to full dose with similar low annual bleeding rates. Further work is progressing in this area including a large study in China.
- The seven year data from the Phase 1 / 2 trial with the factor VIII gene therapy, now licensed as Roctavian, showed an average factor VIII expression of 16% with a median value of 10.3%.

Debbie Greene's highlights from the Congress

'Pain and People with Bleeding Disorders in the New Era'
In this session, Robert Ucero-Lozana from Madrid and Rebecca S. Schaffer from the USA spoke about the active role of the patient in the control and knowledge of pain and the reality of living with pain. Some interesting points were:

- People with bleeding disorders (PWBD) suffer a high prevalence of pain, to the point where it can interfere with their daily lives.
- The consequences of pain can lead to muscle guarding, muscle spasm/inflammation, restricted movement and muscle weakness. This in turn can cause feelings of anger, frustration, depression and helplessness for the person with a bleeding disorder.
- Some PWBD find it difficult to distinguish between acute and chronic pain, which can cause patients to be confused as to the cause of their pain i.e. a bleed or arthropathy.
- The brain plays a major role in the effect of pain on the body and emotions effect how patients feel pain, so this must be taken into account when managing pain.
- The patient's emotional state must be taken into consideration; If a patient feels they are not being listened to, this will have a negative effect on them.

'Sports-related Injuries'
This session addressed sport-related injuries in people with bleeding disorders. It covered the relationship between sports participation and injuries. It provided insights into preventive measures that can be taken to reduce the likelihood of an injury and looked at the balance between muscle strengthening and the potential bleeding risks associated with such activities.

Olav Versloot from the Netherlands spoke about sports participation and injuries and how exercise is known to improve pain management and increase movement range. Due to joint damage and bleeds, physical fitness is affected, with reduced coordination, endurance, flexibility and strength.

It was also mentioned that it is known that children with haemophilia achieve a better quality of life by participating in sports compared to adults. Current treatments allow children with bleeding disorders to participate in sport and physical activity more than any other generation of PWBD and this has a very positive effect on self-esteem and reducing anxiety.

This session concluded by exploring musculo-skeletal challenges in the context of novel therapies, the importance of finding a balance between treatment and maintaining physical activity and a successful return to play for the person with the bleeding disorder.

'Game-Changing Health - Bridging New Treatments, Sports and Everyday Living'
This was a very interesting session and looked at the perspectives of athletes navigating different treatment landscapes. I was particularly interested to listen to Clive Smith from the haemophilia organisation in the UK, who gave an excellent talk from a patient perspective. Clive has severe haemophilia A and is a UK Haemophilia Society trustee. Clive was diagnosed at birth and was taking on-demand treatment until the age of nine years when he started prophylaxis. His worst bleeds were between the ages of 7-10 years and resulted in him often having to use crutches and a wheelchair, during which time he was only able to walk about one week out of every four. Once on prophylaxis, Clive slowly began to play sports such as football, basketball and swimming from between the ages of 10-16 years. At the age of 24 he took up running, participating in a 10km race in July 2007, after which he could not walk for three weeks. He stopped running and when he returned to it, it took him 18 months to build up again. Clive took part in his first triathlon in 2010, and has since completed the Ironman challenge. Clive stressed to do this level of physical activity you always have to be open and honest with your consultant and your physiotherapist about the activity you are doing and the level of training you are undertaking to work together to prevent bleeds. Clive lives by the motto 'It always seems impossible until it is done". (Nelson Mandela)

'Oral Health & Quality of Life'
Lochana Nanayakkara from the Royal London Hospital in the UK chaired this very interesting session and Zikra A. Alkhayal from Saudi Arabia gave a very good talk that explored the multifaceted significance of oral health for people with bleeding disorders. She addressed the anxiety surrounding dental procedures and the potential risks for severe bleeds and surgery and also highlighted the importance of education for both the patients and the healthcare workers. She also stressed the importance of oral health for people with bleeding disorders and stated that if your mouth is bleeding, it is not because of your bleeding disorder, but because of bad oral health, which can be easily rectified. She also discussed the difficulties with oral health and the negative impact it has on everyday life, including on intimacy. She mentioned that in the past, dentists and haematologists had not communicated effectively and that combined care would benefit them both, as well as benefiting the patients. She spoke about the issues she sees in her clinic and highlighted that it is important that everyone includes dental hygiene as part of daily care.

'Transitioning Through Life Stages for Young Adults with Bleeding Disorders'
This session looked at the psychosocial challenges for young adults who are transitioning into adult care. Some of the barriers to successful transition were parental anxiety and dependence on parents.

The main factors to be considered when thinking about transition for young adults are:

- Access to comprehensive treatment
- Family and community support
- Psychosocial resources
- The social and cultural environment

It was stressed that access to comprehensive treatment is important when transitioning to adult care as this provides psychosocial support and more. Family and community also play an important role at this stage. The patient's own psychosocial resources are again an important factor to consider when judging a patient's readiness to transition. Are they mature enough to become independent in their care? Is there professional help available to help them to cope effectively? Lastly, another important factor to consider is the person's social and cultural environment. Some cultures encourage independence at a younger age to others. Some cultures may struggle to acknowledge or accept the condition, which may led to issues with disclosure. This too will affect a young adults readiness for transition.

From our perspective in the IHS, we know that transition may cause feelings of anxiety around meeting a new team of healthcare professionals and new hospital. The process of planning transition can be daunting for the young adult with a bleeding disorder. It is important to know that healthcare professionals and the IHS are here to support and guide young adults and their parents, throughout this process. The transition to adulthood and adult services involves major changes for all involved. However, it is important to remember that moving on to

an adult treatment centre is one of the final steps towards independence and empowering young people in the management of their own bleeding disorder. It is a crucial step as they embark on their exciting journey into adulthood.

Nina Storey's highlights from the Congress

'Women with Bleeding Disorders: Guidelines for a (True) Comprehensive Approach'
Two speakers gave their personal stories during this session, highlighting the importance of early diagnosis for women with bleeding disorders. Christina Vosl is from Austria and has factor I deficiency. She was three years old when she started on prophylaxis and as a result of her early treatment she never had a heavy menstrual period and later she took the oestrogen only pill to stop her periods. Christina believes it is essential for a gynaecologist to be part of the comprehensive care team in the treatment centre, as women/girls need to have an ongoing relationship with a gynaecologist. Christina believes it is the task of the haematologists to find a gynaecologist to work with them.

On the other hand, Latifa Lamhene, from Algeria, has factor V & factor VIII deficiency and was only diagnosed when she was 19 years old. In 2001, at the age of 22 years of age, Latifa had an abdominal bleed from a burst ovarian cyst. She had emergency surgery, but was not given factor concentrate. She experienced an absence of care and treatment because it was believed that girls could not have haemophilia. Latifa started extensive research on haemophilia to understand her condition and contacted a haematologist. She then had her first appointment in 2011. Over time, Latifa has had frequent haemorrhagic ovarian cysts. She was treated with factor VIII concentrate only during a bleed and when having surgeries she was given fresh frozen plasma. Using this made her feel scared because of blood born viruses, but she had no choice. She is now on prophylaxis three times a week and also takes the contraceptive pill. Both Christina and Latifa believe that things need to change for women and girls with bleeding disorders (WGBD). National Member Organisations (NMOs) need to educate WGBD and give them the confidence to speak out about their own health.

Dr. Roshni Kulkarni from the USA and Dr. Caroline Malcomson from Canada then spoke about the importance of early diagnosis for WGBD to ensure the effective management of their bleeding disorder. Some points I took from this were:

- A study showed the average age of diagnosis is 22 years for WGBD.
- One in five women/girls experience heavy menstrual bleeding. One in four of these will have a bleeding disorder.
- It is important to include WGBD in bleeding disorder registries, because numbers are power when advocating for this group.

'Quality of Life – Intimacy and Sexuality Through the Lifespan'
Argentinian psychologist and sexologist Maria Victoria Hernandez gave the first presentation of this session. Some interesting findings from the presentation were:

- 40-50% of PWBD report that their sexual quality of life is negatively affected.
- Sexual health needs to be included in comprehensive care and in future research.
- Chronic pain, haemophiliac arthropathy (reported in 50% of adult patients) and certain drugs can cause reduced sexual desire.
- Psychological aspects cause a mental burden. It is common to fear bleeds and pain, to feel stress, anxiety and depression, which affects the PWBD and their partner.
- Physical changes as we age impact our body image and self-esteem. As we age there is a decrease in sexual hormones. Ageing has an impact on intimacy. Older adults and people with disabilities are not seen as sexual beings, but everyone has the right to sexuality. New types of intimacy should be explored at older ages.

Greig Blamey, a consultant physiotherapist from Canada, gave the next presentation. Some interesting information from his presentation was:

- In a communication study in 2013 of over 260 healthcare providers, 96% considered sexuality to be a relevant topic; However, 71% reported they never raised the topic with patients and 88% gave the patient the responsibility to initiate the discussion.
- Sexual health should be addressed consistently by healthcare providers.
- The impact of non-replacement therapies and consistent factor levels, as well as bleeding history and existing damage, remain relevant factors to consider.
- Many sexual positions require sustained end-range positioning under repetitive force and require muscles to generate force in sub-optimal positions. Choosing a position that matches your body and your partner's body, while taking the strain from target joints, is advisable.

Diane Standish from the USA discussed building healthy relationships and emotional intimacy. Emotional intimacy includes closeness, mutual respect, empathy, honesty and trust. As adolescents (ages 12-18) feelings of passion are high and intimacy are low. During early adulthood (late teens – early 30s), there is less jealousy, more control and fewer negative interactions, which lead to intimacy, independence and finding your identity. Middle age (ages 40-60) relationship priorities include security, loyalty and mutual emotional interests. In late adulthood (age 60+) people value consistency and familiarity, intellectual and spiritual intimacy, while caring for a partner with a chronic disease can place demands on intimacy. Everyone can fall in and out of emotional intimacy depending on what's going on in their lives, such as work commitments, responsibilities, children, changing priorities and enough spare time. Everyone has different needs and expectations of intimacy. Questions to reflect on are:

1. Do I bring my best self to my interactions with my partner or do I hold back?
2. Does my fear of loss and abandonment cloud my perspective and prevent me from sharing my authentic self?
3. Do I feel comfortable asking for what I need and allowing myself to be vulnerable or do I tend to use stonewalling (shut down or distance myself)?
4. Do I possess self-love and expect to be loved and respected?

Emotional intimacy changes over the course of a relationship, but both practical and psychological obstacles can be overcome. Creating and maintaining emotional intimacy requires self-reflection and mutual effort and courage.

'Intimacy for Women with Bleeding Disorders'

Lauren Phillips from New Zealand shared her personal story about navigating sex and intimacy with von Willebrand disorder. Lauren had her first sexual experience in her mid teens. She had a long term boyfriend, but this ended when he told her he 'didn't want to have a child that had something wrong with them'. Single again, she sought intimacy in the wrong places due to her lack of self esteem. Lauren had the Mirena coil fitted when she was 22 years old, but this stopped working after nine months. While she experienced severe pain during her period, she also experienced occasional pain during sex. She sometimes passed out after sex and would bleed severely. All this had a negative impact on her and it was only when she met her husband that things started to turn around. She began counselling and learnt that she was seeking intimacy due to feeling like there was something wrong with her. She learnt to be okay with the person she was and began to rebuild her confidence. With the support of her husband, she learned what real intimacy felt like. This included laughter and open and honest communication. Even with a loving and understanding partner, she still finds intimacy difficult. She pointed out that being able to talk about what feels good and what doesn't, before, during and after sex, is important, and especially to understand what sexual positions or sexual acts will cause a bleed. Lauren used a menstrual cup to avoid visible bleeding and recommends using lots of lubrication to prevent vaginal tears. Navigating intimacy as a woman with a bleeding disorder (WBD) successfully is largely dependant on the way you feel about yourself and your bleeding disorder. Having a caring partner with whom you can communicate your wants, needs and desires is crucial. It is also important to have support from other women. It is the role of parents and healthcare practitioners to ensure that the physical aspects of intimacy are discussed, as well as to ensure that girls feel confident in themselves and in their bleeding disorder.

WBD face many challenges in intimacy, such as low self-esteem/body image, pain during intercourse, fear of bleeding, bruising or injury during sexual activity. 40% of women report bleeds caused by sexual intercourse. They also report emotional distress and relationship strain mainly due to the inability to communicate with their partner, lack of awareness and lack of support from healthcare providers.

A study carried out showed that heavy menstrual bleeding affected all aspects of life, including work, education and hobbies. It also had a negative impact on mental health, contributing to mood changes, anxiety and depression. It showed the experience of WBD is negatively affected by lack of support from health providers, underplaying the impact of bleeding disorders on daily life and emotional suffering. Unfortunately, when reviewed in 2020, these issues were still being reported.

Dan McIntyre's highlights from the Congress

'Pain Management'
The key takeaways for me was that the understanding of pain in haemophilia remains limited, is poorly managed in almost 50% of cases and is influenced by geography, socio-economics and expertise. An interesting speaker (a physiotherapist) felt that "PWBD who are active in sports should be assessed more often than every six months, probably every three months and have variable visits for monitoring with ultrasound to see changes in the soft tissue/synovial, as biomarkers are not as efficient as ultrasound".

'Longterm Joint Health: From Childhood to Adulthood'
The general view at this session was that MRI and X-ray as well as physical examination was not as efficient as ultrasound in detecting bleeds in the synovial and soft tissue. This session also included ageing with joint restrictions and covered physical therapy/surgical interventions, use of pain relievers (short term use of NSAIDS) and adaptations to the home to reduce pain and increase function.

'How to go from Caregiver to Patient'
This was an interesting and thought-provoking session for me as a male haemophiliac. This was related to the role of women who are mainly seen as carriers (caregiver) and not as patients (having a bleeding disorder). However, women also experience bleeding and can be dismissed and minimised by health care providers and encounter diagnostic delays or misdiagnosis.

Suzanne O'Callaghan from the Haemophilia Foundation of Australia (HFA) gave a wonderful presentation on this subject about 'misconceptions and barriers' where she discussed the following points:

- Lack of up-to-date knowledge – women can have bleeding disorders as well as carrying them.
- Symptoms put down to gynaecological problems.
- Family perception - this bleeding is 'normal'.
- An awkward discussion – taboos.
- Hard to advocate when feeling ill, lacking energy.

She gave a good resource to check out on the HFA: https://www.haemophilia.org.au/bleeding-disorders/women-with-bleeding-disorders/the-female-factors/

John Stack's highlights from the Congress

'Longterm Joint Health Monitoring in Haemophilia'
The first presenter, Nihal Bakeer, discussed 'Point of Care Musculo-Skeletal (MSK) Ultrasound'. It was an interesting presentation in that it discussed the use of ultrasound scans carried out by people other than radiographers. The approach is that a simplified interpretation may highlight issues of concern. The presenter suggested that an ultrasound scoring system should be developed using a tiered approach for further intervention if required. It is not certain if ultrasound results from this process should influence treatment protocols as there is not enough data and ultrasound is not guaranteed to detect everything of significance. A final question by the presenter was whether ultrasound results are sensitive to changes in prophylactic therapy.

The final presenter in this section, Cindy Bailey, discussed ageing with a bleeding disorder and musculoskeletal challenges in the elderly. She first pointed out that it is inconclusive if markers in the blood are related to MSK deformities. The majority of her presentation related to the protocol for engaging with elderly patients with bleeding disorders:

- She emphasised that the process should follow a shared decision-making approach and prioritise the patient's personal goals, which are often associated with reduction in pain and increase in function. Some goals are not typical, such as the decision to have a knee fusion.
- Lowering the risk of falls has been identified as an important goal.
- She highlighted the importance of a well-designed and implemented and age-appropriate strength and conditioning programme that prioritises functional training. Functional training is very specific to the individual, such as the case of functioning with a fused elbow and performing activities of daily living.
- She also highlighted the need for adaptive equipment and/or adaptive homes for some patients.

'Capacity Building, Advocacy'
The first speaker, Maria Santaella, presented the results of a study of outreach activities across countries of varying economic output. In particular, she looked at the use of different methods of patient outreach activities in identifying people with bleeding disorders, ranging from print materials, use of mass media, integrated outreach and even door-to-door calls in multiple countries. She grouped the results into four categories (low income, lower-middle income, upper-middle income and high income). It was interesting to see the different methods used based on income. Ultimately, she concluded that successful patient outreach requires a multi-stakeholder approach, including government agencies.

The second speaker, Amanda Brito del Pino Mauro, talked about how she used storytelling to dramatically improve haemophilia care in her country, Uruguay. Interestingly, Uruguay is deemed to be a wealthy country by South American standards, yet haemophilia care was almost non-existent. She talked about establishing a specialised volunteer advocacy team and developing a unique storytelling strategy. She highlighted that data alone is not enough to convince governments to improve the standard of care for rare diseases; it is important to tell the 'story of pain'. She said that she could not underestimate the importance of the process. Her son, who has severe haemophilia, wrote a letter to their President explaining that, due to his haemophilia, he could not play soccer with his friends. In Uruguay, soccer is everything. The letter went viral. She emphasised that the letter was the final part of an advocacy strategy. As a result, 56 patients were able to change their treatment to prophylaxis, including her son. Public policy was also changed. She concluded that structural change is possible through advocacy.

'Use of Technologies for Patients with Bleeding Disorders'
The first presenter looked at the use of virtual reality in the treatment of chronic pain. This is in the context of non-pharmacological pain management. The presenter, in his study, used virtual reality technologies for retraining the brain to recognise chronic pain for what it is. He discussed how people with chronic pain can often become desensitised to it as a result of its permanent presence, meaning that they ignore or no longer recognise symptoms associated with bleeds. He argued that virtual reality therapies could complement or substitute physiotherapy where no or minimal physiotherapy is available.

A second aspect to this presentation was the use of technology for sports activities. An app called WAPPS-HEMO was demonstrated, which graphs factor levels and can

show the patient his current factor level. This could be useful prior to the commencement of physical activity. This could ensure sufficient factor level at the time of playing sport. The presenter also highlighted that fitness trackers could be useful for tracking physical activity levels amongst people with haemophilia. It is important to note, however, that some off-the-shelf activity trackers tend to over-estimate physical activity.

'Quality of Life/Outcome Research'
One of the speakers of the session, Marko Marinic from Croatia, looked at co-morbidities in patients with haemophilia. This was research carried out in Croatia. This research looked at a broad range of co-morbidities and determined their extent in people with haemophlia. All co-morbidities, except for BMI, correlated with age. Co-morbidities were more prevalent in people with hepatitis B or C infection and even higher for people with severe haemophilia. Perhaps the most interesting outcome from this research is that people with haemophilia have a higher life satisfaction index than the general population in Croatia. Several reasons for this were proposed, including the standard of care in Croatia, social connections and the disability paradox.

'Unresolved Issues in Haemostasis in Haemophilia'
Robert Sidonio gave a presentation on muscular haematomas in patients using Emicizumab. He mentioned that there will be papers on the role of sports being published soon. Robert's main concern is complacency amongst patients; In other words, patients on Emicizumab assume they are not bleeding because they are taking that product. Therefore, both increased monitoring and patient education are required to ensure that patients are more aware of bleeding potential.

During the Q&A session, Dr. Beatrice Nolan stated that she has observed significant bleeds in patients taking Emicizumab and gave examples of paediatric patients experiencing bleeds in the iliopsoas (two cases), buttock and rotator cuff (one case of each). Dr. Nolan stressed that both patients with iliopsoas bleeds had not had bleeds in many years. These two bleeds occurred after the patients transferred to Emicizumab and were no longer in the introductory stage.

'Outcomes and Outcome Measures'
Victor Jiménez-Yuste discussed joint health assessments in the era of new treatments. He notes that only 80% of patients have pristine joints (his population are children who have grown up on prophylaxis) suggesting that prophylaxis on its own is not enough to guarantee excellent joint health. He commented that, while annual bleed rate (ABR) is low, and even lower for novel therapies, ABR does not detect sub-clinical bleeds. He stated that MRI is the gold standard for joint assessment, but acknowledged that it is expensive and not always practical or available. He proposed that Point of Care Ultrasound (POCUS) is a useful tool that complements physical examination. This is a modified, lower standard of detection, form of ultrasound that can be completed by medical professionals other than radiologists/radiographers. He proposed that in future, POCUS could be made available for home use where patients themselves can scan their joints and send the images to a specialist, AI, or some form of machine learning. While this might sound ambitious, or even unrealistic, he stressed that the most dangerous phrase is "we have always done it this way". He states that we need to think about how we look for trauma, and study alternative methods, such as biochemical markers.

Dates for your Diary, 2024

July
Von Willebrand & Rare Bleeding Disorders Information Day
Date: Saturday 20th July
Venue: The Grand Hotel, Malahide, Dublin

September
Mild Haemophilia Information Day
Date: Saturday 14th September
Venue: The Grand Hotel, Malahide, Dublin

October
October Members' Conference
Dates: Friday - Sunday 18th - 20th October
Venue: Midlands Park Hotel, Portlaoise

Ballyhea Tractor Run 2024

My Dad, Con Walsh, set up the Ballyhea Tractor Run in 2017 in aid of Marymount Hospice and Milford Hospice after his brother died of cancer. To see the crowds each year that gather to either take part in the Tractor Run or simply spectate, would make one think it was much bigger than a small village in North Cork. The support has always been amazing. When our son, James, was diagnosed with severe haemophilia B in May 2023, at 11 months old, my Dad immediately decided to include the Irish Haemophilia Society in the next Ballyhea Tractor Run.

The Irish Haemophilia Society have been a huge support to my family and I as we navigated the last year of understanding, coping with and treating James's haemophilia in the best way. Everyone who works at the IHS is always so friendly, kind and supportive. The apartment facility has been a lifeline several times for us as we do not live close to Crumlin Hospital and the Parents Conference we attended in July 2023 opened our eyes to the amazing community there is for families living with bleeding disorders.

So this year, on the 7th January 2024, a beautiful, frosty Sunday morning, the Ballyhea Tractor Run kicked off for the 6th year, this year proudly including the Irish Haemophilia Society. Hundreds of people looked on as almost 100 tractors/ trucks/motorbikes and vintage vehicles travelled almost 16 miles from Ballyhea to Charleville to Kilmallock and back.

It is very emotional to see so many people of all ages taking time to support such worthy causes. I felt it was also a great event to raise awareness for the IHS as not many people have heard about bleeding disorders, not to mention the supportive networks out there for such people and their families.

Proceeds were split equally and €1,900 was donated to the Irish Haemophilia Society. I am so proud and grateful to all who helped make the Ballyhea Tractor Run a success, especially my family. A very, very special thank you has to go, in particular, to my Dad, who would forever jump through hoops to help his grandson James. Roll on Ballyhea Tractor Run 2025… all are welcome!!

Michelle McKeon, IHS Member

Prof. O'Donnell Receives Award for Contribution to Haemophilia Care

The Brian O'Mahony Award for Outstanding Contribution to Haemophilia Care in Ireland for 2023 was awarded to Professor James O'Donnell from the National Coagulation Centre (NCC) at a reception at the Society office on May 8th.

Prof. O Donnell is a professor of vascular biology at the Royal College of Surgeons in Ireland and the director of their vascular biology department. He is a professor of haematology at Trinity College Dublin and a consultant haematologist at the NCC at St. James's Hospital since 2005.

James is a world renowned researcher into and authority on von Willebrand disorder. He has led the Low Von Willebrand in Ireland Cohort (LoVIC) study and the impactful Irish Personalised Approach to the Treatment of Haemophilia (IPATH) study. As a clinician, James is highly valued by his patients for his empathy, compassion, knowledge and practical approach to problem solving. James has collaborated with the Society for many years in his research, on von Willebrand awareness and as an impactful speaker at our conferences and events.

Brian O'Mahony, Chief Executive

Strategic Planning

The Irish Haemophilia Society will be producing a new strategic plan for the period 2025 to 2028 by the end of this year. In early December, the board and staff will meet for an intensive two day work session where we will outline the key goals, objectives and strategies which will form the new strategic plan. Prior to this we are keen to ascertain the views of members from different demographics in relation to the objectives, strategies and actions we should be taking as an organisation in the next four years. To this end, we will be carrying out surveys at all our events throughout 2024. We will also be utilising mail surveys and online surveys with members later this year to ascertain your views. We will also be separately gathering the views of our youth members, our volunteers and of key healthcare workers in the centres. We are cognisant that we have to provide services and support to all members – including those with von Willebrand disorder, rare bleeding disorders and platelet disorders. We are living in an era of unparalleled therapeutic advances in haemophilia, which we hope will encompass von Willebrand and rare breed disorders in the coming years. While being fully cognisant and prepared for this we also need to look at the structures and function of our comprehensive care services and our ongoing interaction with them to ensure that they are optimised to meet the needs of the patient population. We look forward to your positive engagement with us in this process.

Brian O'Mahony, Chief Executive

Von Willebrand Disorder & Rare Bleeding Disorders Information Day

Date: 20th July, 2024
Venue: The Grand Hotel, Malahide, Dublin

Our annual information day for von Willebrand disorder (VWD) is approaching again and this year we are incorporating rare bleeding disorders (RBDs) into the day. We are conscious that we haven't had an information day for RBDs and want to include all bleeding disorders in our outreach efforts.

We are lucky to have Professor James O'Donnell and Dr. Beatrice Nolan speaking at this year's event. We will have a session about the changing treatment landscape for people with VWD and we will also have one on RBDs and developments in their treatments. There will also be a panel discussion and lots of opportunities to ask the doctors any questions you may have.

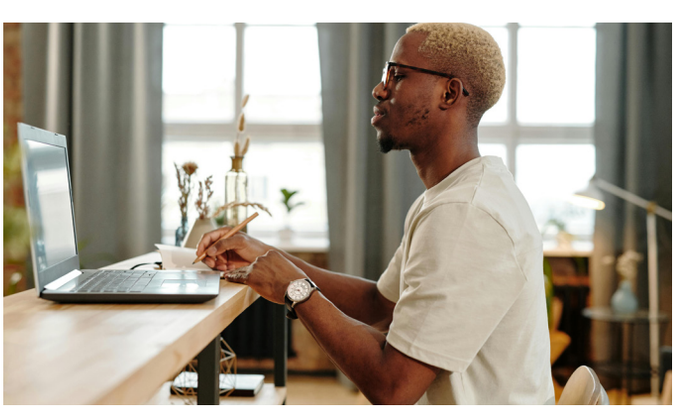
Please email info@haemophilia.ie or contact Louise in the office on 01 657 9900 to register for free.

Physio-Exercise & Pilates Classes

Want to get fit from the comfort of your own home? The IHS continues to hold weekly online physio and pilates exercise classes, and there's always room for more members to join! We started our physio-exercises in 2020 and since they were going so well, we wanted to expand to include a different kind of class - pilates.

Our physio-exercise classes take place every Tuesday evening from 7.30-8.30. These are led by physiotherapist Mark McGowan.

Our pilates classes take place every Wednesday evening from 7-8. These are led by physiotherapist Carly Blackburn.



Educational Grants

This year, our educational grant applications will open at the end of July and will close in October.

Our educational grants are given to people with haemophilia or related bleeding disorders and/or to their immediate family members who go on to do a post second level educational course. The grants provide students with supports for all the expenses of college.

For more information on the grants, see our website under Services & Support. Please an eye out for more information in our Mailchimp and on our website!

Irish Haemophilia Society

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