IRISH HAEMOPHILIA SOCIETY LTD
Cumann Haemifile Na hEireann

Haemophilia.ie

Magazine of the Irish Haemophilia Society

Representing people in Ireland with haemophilia, von Willebrands & related bleeding disorders

SCAN ME

Autumn 2024 Edition

FROM THE EDITOR

Hello members! After a rather disappointing summer we are heading into the season of pretty colours, cooler temperatures and spooky creatures. I hope you're all looking forward to this period as much as I am!

We have an edition packed with diverse and interesting articles, which I hope you will enjoy reading. Brian O'Mahony's report will update members on the new and controversial ISTH guidelines, as well as providing information about a potential shortage of the product NovoSeven.

Afterwards, we have an appreciation of Dr. Barry Harrington, a dentist many members will remember as being the first to develop quality dental care for people with haemophilia at a time when many dentists refused to take them on as patients.

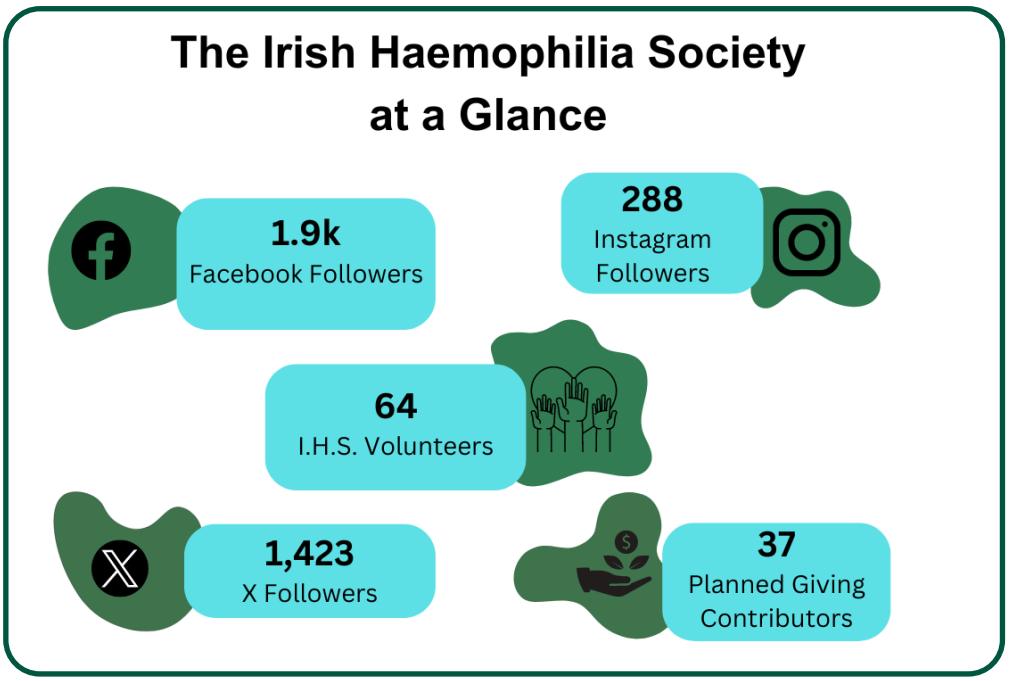
Another highlight of this edition is an interview I conducted with Rana Saifi, from WFH. Rana fills us in on her exciting and meaningful career working with haemophilia societies and other stakeholders across the region of the Middle East.

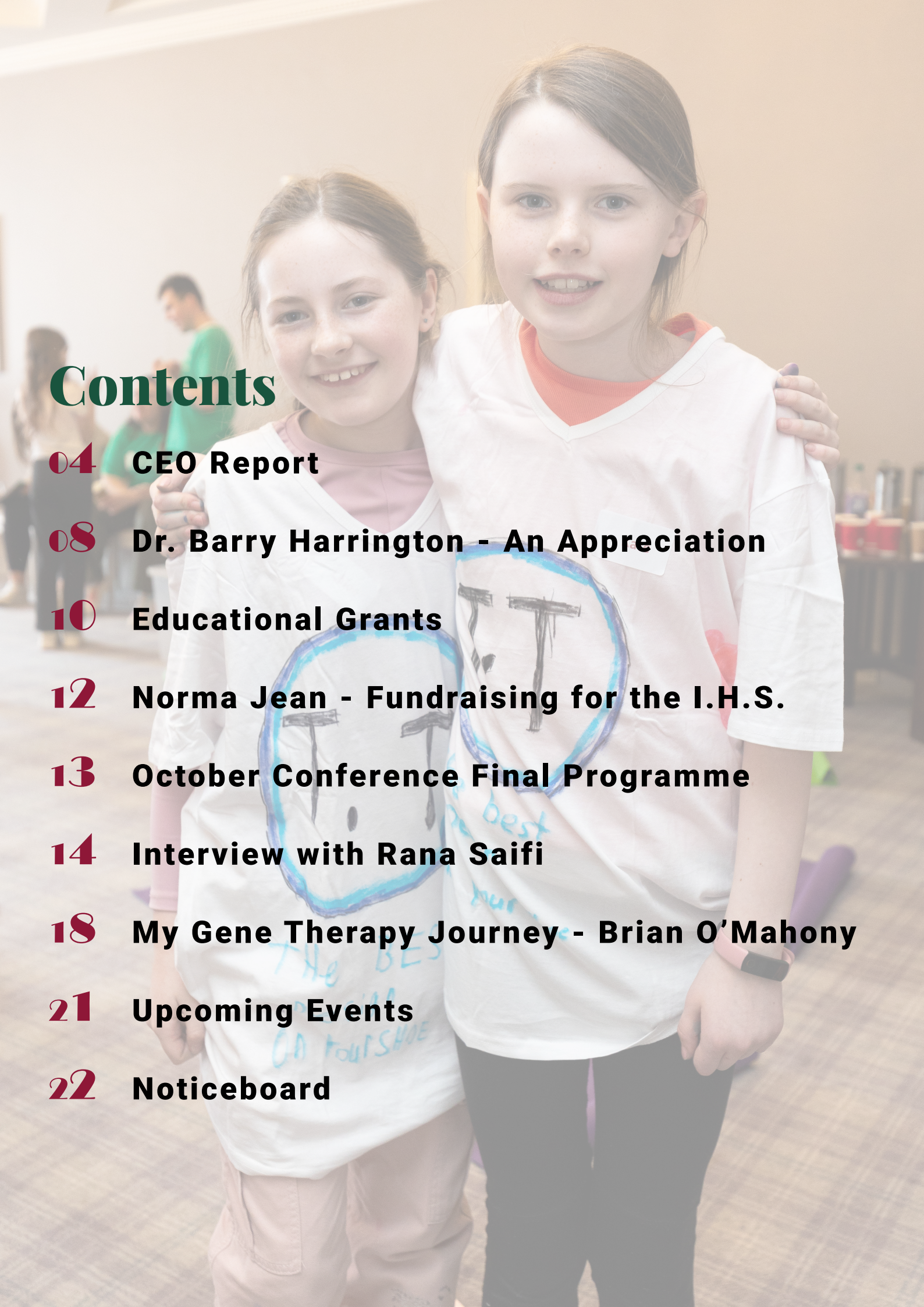
The next article is Brian's gene therapy journey, in which he shares his thinking behind joining the clinical trial for haemophilia gene therapy and recounts in detail what was involved before, during and after infusion day. This offers great insight into one experience of receiving haemophilia gene therapy.

Along with these articles, we have information about our educational grants, for which we are still accepting applications, upcoming events and our Hyde Square apartments. There is also a section of notices at the back of the magazine which members may find informative.

I hope you enjoy this magazine!

Roisin Burbridge, Publications, Website & Social Media Coordinator





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CEO REPORT

WHO Essential Medicines List

In a previous newsletter, I discussed the incorrect and damaging World Health Organisation (WHO) Essential Medicines List (EML) review for haemophilia. This review last year added pathogen reduced cryoprecipitate to the core EML (list of vital medicines which should be used based on safety, efficacy and affordability) with untreated cryoprecipitate as an alternate. Safe and effective plasma derived factor concentrates were kept on the secondary complementary list. Recombinant factor concentrates, which have now been on the market for 30 years, are not included on the list and neither are innovations from the past 10 years including extended half-life recombinant factors or Emicizumab. As part of a World Federation of Hemophilia (WFH) delegation, I met the WHO in Geneva in May to outline our very deep concerns regarding this totally unacceptable situation which goes against any logical approach to safety, efficacy or economics. Their decision-making process was deeply flawed and incorrect. We have recently published a serious critique of this situation in the Journal Lancet Haematology.

I have been working with WFH to reverse this decision. Formal submissions are now being prepared for the removal of cryoprecipitate from the list, the elevation of factor concentrates to the core list and the addition, hopefully, of recombinant factors, and Emicizumab. We will keep you informed of developments. The list is next updated in 2025 and submissions are a slow and laborious process. Bizarrely, WHO have no procedure to remove dangerous therapies (such as untreated cryoprecipitate) from the list outside of these formal reviews.

ISTH Haemophilia Guidelines

A separate issue which is causing deep concern in the community is the recently published Haemophilia Guidelines from the International Society of Thrombosis and Haemostasis (ISTH). These guidelines followed a very strictly applied methodology called GRADE which places a lot of emphasis on randomised control trials (RCTs). This type of clinical trial is very common when looking at therapies for common conditions but more difficult and problematic with rare diseases like haemophilia. If used strictly, this methodology can result in drawing the conclusion that there is very low certainty of evidence for the use of most therapeutic options as haemophilia is a rare disease. This type of approach was previously used in published von Willebrand guidelines but applied with a clearer appreciation of the difficulties and therefore also used clinical expert opinion and patient relevant outcomes in their deliberations.

These ISTH guidelines have resulted in 13 recommendations. These include a recommendation on prophylaxis for factor VIII (FVIII) deficiency with Emicizumab or FVIII concentrates and a separate recommendation on prophylaxis for FVIII deficiency with either standard or extended half-life factor concentrates. These two are seemingly duplicative and confusing. For factor IX (FIX) deficiency, a recommendation is that prophylaxis be carried out with plasma derived, standard recombinant or extended half-life FIX concentrate. This encompasses all the possible therapies and ignores the very significant benefits of extended half-life FIX which provides higher trough levels, more protection from bleeding and less frequent infusion. These Guidelines do not distinguish clearly between the efficacy of standard factor concentrates, extended half-life concentrates or the mimetic Emicizumab. They clearly contradict the evidence-based Guidelines produced by WFH in 2020 and


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COMMENT · Volume 11, Issue 9, E638-E640, September 2024

Risk of harm to people with haemophilia from the 2023 WHO Essential Medicines List

Glenn F Pierce [a] · Brian O'Mahony [a,b] · Radoslaw Kaczmarek [a,c] · Mark W Skinner [a,d,e] · Mike Makris [f] · Flora Peyvandi [g,h], et al. Show more

Affiliations & Notes Article Info

"The Essential Medicines List is of great importance because many governments rely on it for their national Essentials Medicines Lists. If WHO does not support using disease-specific treatment guidelines, it becomes an unreliable source of information for both governments and health-care providers."

"Risk of Harm to people with Haemophilia from the 2023 WHO Essential Medicines List", The Lancet Haematology, September 2024

the recommendations from the Council of Europe in the same year which strongly recommended and led to higher factor trough levels (recommended trough levels of at least 3% to 5% as opposed to the previous standard of 1%) and greater protection from bleeding for people with haemophilia. They also contradict the established actual clinical practice in the countries of several of the co-authors of these guidelines.

A coalition of concern including WFH, the European Haemophilia Consortium (EHC), leaders of the European doctors group EAHAD, the National Bleeding Disorders Foundation and Coalition for Hemophilia B in the USA, the clinical haemophilia groups from Asia Pacific and Latin America, have all expressed their deep concerns both about the methodology used and more importantly, the potential harm these guidelines could do in relation to access to treatment for people with haemophilia in many medium or low-income countries. There is the real possibility that health ministries may use the fact that ISTH state that 10 of the 13 recommendations are based on very low certainty of evidence to avoid procurement of safer, better therapies and choose instead to purchase cheaper, older and less effective products. In addition to impacting developing or emerging countries the guidelines may possibly impact some high income countries including the USA where some payers, concerned only about cost, and not efficacy of treatment, may use these guidelines as an excuse to revert to older, less effective and less expensive therapies.

Papers have now been submitted to scientific journals co-authored by almost 50 global clinical and patient leaders in haemophilia setting out our deep concern about these guidelines. The organisations concerned will continue to advocate to mitigate any harm these guidelines may cause. It is frankly disappointing that so much time and effort has had to be expended on battling issues such as these ISTH guidelines and the WHO essential medicines list where, rather than moving the community forward, we are fighting to prevent damage to prevailing treatment standards.

Treatment availability developments

There have been two significant developments in recent weeks in relation to treatment availability. Biomarin, the manufacturer of the only currently licenced FVIII gene therapy marketed as Roctavian, have announced that they are now limiting availability of this product to the United States, Germany and Italy. Roctavian has been licenced by the FDA in the USA and the EMA. However, agreements on reimbursement have been slower and sales of the FVIII gene therapy have been much lower than the company initially anticipated. To date, the licensed gene therapy has been sold only in those three countries and the company have now decided to market only in those countries utilising the gene therapy stock they have in hand. This decision may be revisited in the future by the company. There are currently other FVIII gene therapy products in late stage clinical trials by other companies but not yet licensed.

The second issue is a potential shortage of the bypassing agent NovoSeven (FVIIA) manufactured by Novo Nordisk. This is used for treatment of bleeding episodes in people with FVIII or FIX inhibitors in addition to some rare bleeding disorders. The company have informed the global community, and Ireland, of a manufacturing issue which resulted in some vials being underfilled. They are taking steps to remedy this but there is a risk of a supply shortage which may last until the end of 2024. We are fortunate that we have a national system of procurement, supply and stock management and steps have been taken and are being taken by the National Coagulation Centre (NCC) and the other centres to manage any potential shortage and mitigate the situation in a planned way. For those who use NovoSeven, it will continue to be available for acute bleeding episodes, urgent surgery and prophylaxis. Non-urgent surgical procedures which may require treatment with NovoSeven will be deferred. We have directly contacted members who use this treatment and we will keep the community informed as we receive updates.

Clinical webinars

The Society have organised a series of six clinical webinars which started on September 4th. The intended audience are clinicians and healthcare workers both here in Ireland and globally.

The topics and speakers are:

- **Update on Haemophilia A & B** - Professor Niamh O'Connell, Director of the NCC
- **Update on Gene Therapy for Inherited Bleeding Disorders** - Dr. Radoslaw Kaczmarek, Assistant Research Professo of Pediatrics, Gene Therapy and Cell Therapy Laboratory, Indiana University School of Medicine
- **Women and Bleeding Disorders** - Dr. Michelle Lavin, NCC
- **Update on Novel Therapies** - Professor Flora Peyvandi, Director of the Angelo BiancoBonomHaemophilia and Thrombosis Centre in Milan
- **Update on Von Willebrand Disease** - Professor James O'Donnell, NCC
- **Future of Paediatric Care for Children with Inherited Bleeding Disorders** - Dr. Beatrice Nolan, CHI Crumlin

We have been assisted internationally by the WFH, EHC, National Bleeding Disorders Foundation (USA) and the European Association for Haemophilia and Allied Disorders (EAHAD) to advertise the webinars to relevant healthcare workers. The content will be clinical and not focused primarily at people with bleeding disorders, but members who would like to attend are free to do so. You can register free of charge by visiting our website and going to Resources - Webinars. The webinars have been made possible by an unrestricted educational grant from Novo Nordisk but the content of each lecture is entirely at the discretion of our invited expert speakers.

Visit from Jordanian Twinning partners

Since 2019, the Society has been twinned with the Haemophilia and Thalassemia Society from Jordan under the auspices of the WFH organisation twinning programme. The NCC and the centre at Children's Health Ireland (CHI) at Crumlin have also been twinned at the same time with the main haemophilia treatment centres in Jordan. This has allowed a degree of synergy and a co-ordinated approach to assisting with the development of treatment, care and the patient organisation in Jordan. Unfortunately, the Covid pandemic had a restricting impact on our ability to collaborate in person, so several activities took place online.

I am happy to report that three representatives of the patient organisation visited Dublin in July, along with four healthcare workers who were part of the centre twinning work and the WFH programme manager for the Middle East region.

We had two separate working programmes for the week, but these were also co-ordinated. With the involvement of some board members, all of the staff and myself, the Society visit programme included training on finance, outreach, volunteer recruitment and retention, governance, publications, children's programmes, social media and organising events. We also had a productive action planning session where we assisted our Jordanian colleagues to set out their key objectives for the next three-year period. Some of the objectives included having a consistent supply of treatment, adequate home treatment, better treatment options, improving hospital waiting times, advocacy and governance. We organised tours of the comprehensive care centres at St. James's and Crumlin in addition to a tour of the Irish Blood Transfusion Service. There was also an opportunity for the Jordanian team to meet the Syrian boys with haemophilia and their fathers, who have moved to Ireland from Jordan. We also arranged a home visit to one of our members which was very beneficial.

It was a very productive visit and marks the formal end of this twinning programme. We shall, of course, continue to stay in touch and offer advice and support when required and strategic help to Jordan. The visit ended with a lovely evening where they were brought on a tour of Trinity College including an opportunity to see the Book of Kells and visit the new and impressive Book of Kells Experience.

Brian O'Mahony, Chief Executive

Dr. Barry Harringto

Dr. Barry Harrington, who passed away recently, made a very significant contribution to the haemophilia community in Ireland. Barry and his team from the dental hospital took over the haemophilia dental service at St. James's Hospital in the mid 1980s. It is no exaggeration to say that this revolutionised dental care in haemophilia. Prior to that it was very difficult for people with severe haemophilia to access routine dental care. The dental service at the haemophilia treatment centre prior to his arrival was very rudimentary and even simple procedures such as fillings would not be routinely done because of concern about giving dental blocks.

Barry and his team cut through many of the myths surrounding care in haemophilia and the fear that many dentists had in treating people with inherited bleeding disorders. They carried out many procedures and established that this could be done safely without factor concentrates. They worked through a significant backlog at that time and produced a top class dental service for people with haemophilia and related bleeding disorders.

Barry also produced the first ever dental monograph for the World Federation of Hemophilia (WFH). This contribution vastly improved dental care, though the huge positive impact Barry had on the community extended much further.

In 1987 I worked with Barry to carry out a detailed survey of our members who had been impacted by HIV. The results of this survey were used to put together the first submission to the government on the need for recompense for people with haemophilia who had been impacted by HIV and the subsequent advocacy campaign led to the government defeat in the Dail in April 1989. This in turn led to a general election and the establishment of the Haemophilia HIV Trust and the first recompense for those with HIV and their families.

Barry served on the board of the Irish Haemophilia Society for 15 years from 1991 to 2006. When the WFH international congress was held in Dublin in 1996, Barry was chair of the organising committee. That congress was the most successful ever in the history of the WFH to that point with a record international attendance. I know that Barry had not intended to stay on the board of the Irish Haemophilia Society for as long as he did but, as Society Chairman, I was very reluctant to let him leave. His input and advice was always greatly appreciated and constructive. He would often approach a complex problem in a different way from anybody else on the board and would always assist us to think strategically in different ways. He was also a pleasure to work with. He had strong opinions and views, was never afraid of stating them and arguing his case but always in a very constructive and inclusive way. He was caring, empathetic, strategic and he had a great sense of humour. After we reluctantly agreed to Barry stepping down from the board he came back as Chairman of the statutory National Haemophilia Council in 2013 and served in that capacity for six years until 2019.

I worked with Barry on the Society board, on the organising committee for the WFH Congress and on the National Haemophilia Council respectively from the mid 1980s to 2019. I regarded him as a friend, a mentor and a truly wonderful human being. Our deepest sympathy go to his wife Laura and his family but they can perhaps take some consolation that, among his many other achievements, he made a very significant constructive, lasting and positive difference the lives of the entire Irish haemophilia community.

May he rest in peace.

Brian O'Mahony, Chief Executive

on - An Appreciation

EDUCATIONAL GRANTS

The Society offers annual educational grants to people with haemophilia, von Willebrand disorder and other related inherited bleeding disorders, including to a person with carrier status and/or to their immediate family members.

The purpose of these educational grants is to offer financial support to post-second level students to assist them with the extra expenses of their studies. Applications opened on 22nd July and **close on 25th October 2024.**

Types Of Educational Grants

There are four categories of grants:

- Maureen & Jack Downey Educational Grant
- Margaret King Educational Grant
- Father Paddy McGrath Educational Grant
- Michael Davenport Educational Grant

Criteria For Applying

The Maureen & Jack Downey Educational Grant:
- Available to a person with a bleeding disorder, including to a person with carrier status.
- The person must have been accepted on to a post second level course from level 7-9.
- The person must be registered at the National Coagulation Centre at St. James's Hospital in Dublin.

The Margaret King Educational Grant:
- Available to an immediate family member of a person with a bleeding disorder, such as a spouse, child, sibling or parent.
- Carriers with factor levels greater than 40% can also apply for this grant.
- The person applying must be accepted on a post-second level educational course at levels 7 to 9.
- The person with the bleeding disorder must be registered at the National Coagulation Centre at St. James's Hospital in Dublin.

The Father Paddy McGrath Educational Grant:
- Available to a person with a bleeding disorder, including to a person with carrier status who has been accepted onto a post-second level educational course at level 5 or 6.
- Also available to immediate family members who have been accepted onto a post-second level educational course at level 5 or 6.
- The person with the bleeding disorder must be registered at the National Coagulation Centre at St. James's Hospital in Dublin.

The Michael Davenport Educational Grant:
- Available to a person with a bleeding disorder, including to a person with carrier status who has been accepted onto a post-second level educational course at level 7 to 9.
- The person must be a mature student going back into third level education.
- The person with the bleeding disorder must be registered at the National Coagulation Centre at St. James's Hospital in Dublin.

Award Amounts

Maureen & Jack Downey Educational Grant
- First prize €4,000
- Second prize €2,000
- Third prize €1000

Margaret King Educational Grant
- First prize €2,000
- Second prize €1,000
- Third prize €500

Father Paddy McGrath Educational Grant (2 Grants)

1) A person with the bleeding disorder
- First prize €1,000
- Second prize €500
- Third prize €250

2) A family member of a person with the bleeding disorder
- First prize €500

- Second prize €250
- Third prize €125

Michael Davenport Educational Grant
- First prize €3,000

Process of scoring applications

Once the closing date arrives, towards the end of October, a subgroup of three people from the executive board (which cannot include anyone with a family member applying for any of the other grants) meet to consider and score the applications, and make recommendations to the rest of the board regarding recipients. The successful applicants are then notified at the start of November by letter.

Applications are scored on the following:

- The quality of the application.
- The information provided on the application form.
- Involvement in the Irish Haemophilia Society.
- Financial need.
- How many in the family are going to college.
- If the application is a first time application.

Can I apply every year?

Yes, you can apply every year, even if you have already been successful. Please remember that you can only apply to one grant each year.
You can apply online via our website at www.haemophilia.ie. You can also download the application forms from the website, complete them and post the completed forms to the office. If you need further assistance, call the office on 01 657 9900.

The closing date is 25th October 2024.

Hyde Square Apartments

A quick reminder that our apartments at Hyde Square are available to:

- People with haemophilia or related bleeding disorders from outside of Dublin, when attending St. James's Hospital or Children's Health Ireland, Crumlin for treatment, for a hospital appointment or for a review clinic.
- An immediate family member, a spouse, a partner and/or child of the person with haemophilia or related bleeding disorder from outside Dublin, when attending St. James's Hospital or Children's Health Ireland at Crumlin for treatment, for a hospital appointment or for a review clinic, or while a family member is an in-patient.

If you would like more info or to make a booking, please contact the office on 01 657 9900.

A nominal fee of €10.00 per booking, per night will be levied to offset the cost of cleaning and routine maintenance.

VHI Mini Marathon

My little gorgeous baby boy Ozzy was diagnosed with haemophilia B in late March. This was all new to me and my partner Sean. As the weeks went on, I became aware of the amazing charity, the Irish Haemophilia Society, and what they do. My sister-in-law Emma had a great idea to do the mini marathon and try to raise funds. Not only would this give us a push but it would also raise money for a great cause! I set up a go fund me shortly after getting my number for the event and not only was I completely blown away by the generosity of friends and family that donated, the numbers just blew my mind!! We raised over 3,000 euro in the end. This amount is still absolutely insane to me!

Things then took a turn and Ozzy wasn't doing very well getting his treatment in Crumlin. He began to form an inhibitor from his medication and this meant everything was all up in the air. I was sadly then admitted to hospital the weekend of the VHI mini Marathon. This was devastating to me because not only was I so concerned about Ozzy, but I didn't want to let people down. My sister-in-law Emma and mother-in-law Sandra flew the flag for me and did incredible. Not only did they complete the mini marathon but they said the atmosphere and energy was amazing on the day and with Ozzy and myself in mind they said it really gave them a necessary push. In 2025, I really want to complete the VHI marathon myself and to be a lot stronger but most of all I want to make Ozzy proud!

Norma Jean Kelly, I.H.S. member

Thank you so much to Norma Jean, Emma and Sandra for fundraising for us at the I.H.S.!

IRISH HAEMOPHILIA SOCIETY

October Members' Conference

Venue: The Midlands Park Hotel, Portlaoise, Co. Laois
Date: 18-20th October, 2024

Adults Programme

Friday 18th October

6pm - 7pm	Family Fun Time with Face Painting **(Hotel Lobby)**
7pm - 9pm	Buffet Dinner **(Maryborough Suite)**

Saturday 19th October

10am - 11am	**Dental Care (Maryborough Suite 1)** *Chair: Mr. Jim O'Leary, Executive Board, I.H.S.* *Speaker: Dr. Alison Dougall, Dental Consultant, National Coagulation Centre (N.C.C.), St. James's Hospital*
11am - 11.30am	Tea/Coffee Break **(Pre Conference Area)**
11.30am - 12.15pm	**Travel & Moving Abroad (Maryborough Suite 1)** *Speaker: Mr. Robert Flanagan, Outreach, Volunteer & Children's Programmes Coordinator, I.H.S.*
12.15pm - 1pm	**New Therapies – New Opportunities (Maryborough Suite 1)** *Chair: Mr. Brian O'Mahony, Chief Executive, I.H.S.* *Speaker: Dr. Beatrice Nolan, Consultant Haematologist, CHI, Crumlin*
1pm - 2pm	Lunch **(Triog Restaurant)**
2pm - 3.30pm	**Interactive Workshop: Perspectives of living with a Bleeding Disorder (Maryborough Suite 1)** *Facilitator: Mr. Brian O'Mahony* **OR** **Von Willebrand Disorder & Rare Bleeding Disorders (Dunamaisse Suite 4)** *Chair: Ms. Barbara Wynne, Executive Board, I.H.S.* *Speakers: Dr. Michelle Lavin, Consultant Haematologist, N.C.C. & Dr. Beatrice Nolan*
3.30pm - 4pm	Tea/Coffee Break
4pm - 5pm	**Mindfulness for Peace & Wellbeing (Maryborough Suite 1)** *Facilitator: Ms. Mel Taylor, Holistic Life Coach*
7.15pm	Dinner **(Maryborough Suite)**

Sunday 20th October

10am - 11am	**Youth Debate: Life First, Bleeding Disorder Second? (Maryborough Suite 1)** *Chair: Mr. Robert Flanagan* *Debaters: Mr. Gabriel O'Connor & Mr. Jake Phoenix, I.H.S members & volunteers*
11am - 11.30am	Tea/Coffee Break **(Pre Conference Area)**
11.30am - 12pm	**Strategic Plan 2025 - 2029 (Maryborough Suite 1)** *Speaker: Mr. Brian O'Mahony*
12pm - 1pm	**Open Forum (Maryborough Suite 1)** *Panel including board members Mr. John Stack, Mr. Seamus McDonald, Mr. Dan McIntyre & Ms. Hannah Byrne & staff members Mr. Brian O'Mahony & Ms. Debbie Greene*
1pm	Finish & depart

Interview with Rana Saifi

Ms. Rana Saifi is the Regional Manager for the Middle East at the World Federation of Hemophilia (WFH) and was also heavily involved in the Irish Haemophilia Society's recent twinning programme with Jordan.

Below is an interview Roisin Burbridge conducted with Rana this summer.

Tell us a bit about yourself.

I have been working with the WFH since November 2015. I was always interested in international development, and after studying business first, I completed an MSc in this field. My work and expertise is mainly in bilateral collaboration. I was very excited when I joined WFH because this was a new field for me. While I had never heard about haemophilia before joining, my previous experience in international development helped me a lot in this new role. I have learnt a lot since joining WFH, such as how fantastic this community is. It is small, vibrant, tightly knit, brave and capable of doing amazing things for the people that it serves.

Tell us about your role as Regional Manager for the Middle East.

Initially I had responsibility for both the Middle East and Africa regions, but the role was later restructured, and we now have two people to take care of Africa, and I only look after the Middle East. I cover 22 countries, spreading across an array of geographical regions from Pakistan, Afghanistan and Iran to what is classically known as the Middle East to North African countries such as Sudan and Djibouti. We follow the World Health Organisation (WHO) classification for the Eastern Mediterranean region. Due to time and resource limitations, as well as the different country contexts which can at times be unstable, we are not able to engage with all 22 countries equally.

My key role is to engage with the national member organisations (NMOs) in my region on a frequent basis to see what the needs of the community are in each country. In the countries where we implement healthcare development programmes, such as the Path to Access to Care and Treatment (PACT), we can provide systematic assessment and support for capacity building, finance and engagement at the country level through Memoranda of Understanding

(MOUs) with governments. Other countries can participate in the Twinning Programme, as well as various fellowships that NMO members can apply for such as youth leadership, and the Susan Skinner Memorial Fund Fellowship for example. We also have an IHTC fellowship programme targeting healthcare professionals. While some of these fellowships are competitive, we can reach out and support countries in their applications. I have colleagues who manage these programmes, but when we receive applications from the countries that are in my region, I am asked to review them and provide feedback based on what I know about the country.

What would a typical workday look like?

The role of regional manager is unique as one day you may be on calls and follow up with enquiries from the region, and the next day you may be planning for a country visit or preparing for a workshop or a meeting with the ministry of health. Every day is very different. I would often start my day checking my WhatsApp and emails to see what is urgent. I communicate with many people in my region through specific WhatsApp groups that they set up for patients or doctors or for groups that include both lay and medical people at the country level. For more official business, I communicate by email. Following this, I usually have meetings and spend the day making lots of phone calls or joining Zoom meetings to follow up on specific issues, or prepare for country visits. So, a lot of communicating both internally and externally.

I love this part of my job. The most rewarding part of our job is if we feel that we have been able to support an NMO, so that they can in turn support the community, or if we have been able to support a medical professional so that they can support their patients. It is also very rewarding to be able to support the patients to access resources and treatment.

Could you tell us about your role in the twinning programme with Jordan?

On our team, we have a dedicated coordinator for the twinning programme, who coordinates the programme overall. As regional manager, I identify potential partnerships, conduct assessment visits, offer ongoing support and monitor progress as the partnerships advance.

For example, the idea behind the twinning with Jordan came up during a country visit I was making to Amman. I discussed with the Jordanian NMO the idea of them building capacity through a twinning programme. They had already had a twinning partnership in the distant past with another country, but they felt that having another one would be useful. We asked Brian and he said the IHS would be thrilled to take part.

So, my role was to plant the idea of building capacity through a twinning partnership and then to discuss who I thought would be the perfect match and find out whether they would be willing to engage in this partnership. Twinning is a two-way learning process. The exchange is very important. What's also good about twinning is that it doesn't simply end. The relationship between the two teams has been built and they have become colleagues and friends. They continue to communicate and call on each other for future support.

At the same time, the Jordanian Haemophilia Treatment Centre was making very good progress and had a new Haemophilia Treatment Centre director. We thought that to help them and to help improve lay-medical collaboration, the idea of a joint medical twinning would be appropriate. That's how we ended up with a dual twinning partnership – the IHS and Jordanian NMO and the treatment centres in both countries. The reason this worked very well is because in Ireland you already have such a great model of strong lay-medical collaboration between the IHS and the hospitals. That's why it was easy to think along those terms.

Is it rare for a country to have both medical professionals and NMO twinning with the same country?

It happens but it's not a must. At the WFH, the Haemophilia Organisational Twinning (HOT) is one programme and the medical twinning is another programme. Oftentimes the needs of a country do not make it necessary to have both at the same time. They might have a HOT twinning one year and then five or six years later they might think about having an HTC twinning. In Jordan, it was the opportune moment to have both together.

I imagine haemophilia care varies greatly across your region. Could you expand on this?

Yes, it is very different. My region is very diverse, and includes countries that are lower income, lower middle income and higher income. For the countries with more resources, the ability of patients to be accurately diagnosed and to access treatment is generally easy. For countries with lesser resources the possibilities are fewer as well. In those countries, we call on the WFH Humanitarian Aid Programme to supplement some of the needs of the country, combined with systematic advocacy efforts carried out on national level to make access to treatment and care sustainable.

My region has two issues: inequities in terms of treatment in the region as a whole because of resources, and sometimes inequities within the country itself. The WFH vision is "Treatment for All". I therefore prioritise partnering with stakeholders at the country and the regional level to work towards improved access at both levels. For example, in countries where we have direct programmes of support, we try to ensure that the development of treatment guidelines that can be applied throughout the country consistently is a key priority.

The final point I can mention about experience sharing in my region, is that under the umbrella of the WFH, the medical professionals in the region decided to create what they call the Hemophilia Eastern Mediterranean Network: HEMNET. This provides, on a yearly basis, a forum for the doctors from the different countries to come together and discuss their country level contexts and share their expertise. At the WFH, we are closely involved with this forum.

How many of the countries you work with have access to prophylaxis?

Most of them do not have access to prophylaxis. Some countries with lesser resources still have access, such as Tunisia, which has access to low dose prophylaxis and does it effectively. The countries with good resources provide prophylaxis at higher rates. Some countries that are benefiting from the WFH Humanitarian Aid Programme have put a small number of patients on prophylaxis. Our goal is to ensure they all implement the WFH recommendations in this regard.

Do you find that you work more with the lower income countries or equally with the higher income countries?

We try to work with all of them in different ways. We have a PACT programme in three countries: Pakistan, Egypt and Palestine. Palestine's isn't very active at the moment. PACT is a solid 4-5 year partnership programme with three countries, with MOU's signed with the national government in two of them.

We have other models of engagement such as country programmes and we are also able to provide *ad hoc* support to certain countries for specific issues.

How does WFH manage working in war-torn areas?

This is extremely challenging. In some circumstances we are able to support these areas through the WFH Humanitarian Aid Programme, while in others channelling this aid into the country is more difficult, logistically and otherwise. What adds to the complication is if the relevant country is not yet accredited with the WFH. This sometimes turns into a vicious circle: to be accredited we have to conduct an accreditation assessment visit – but because these are unsafe countries to travel to, we are not yet able to complete this assessment. So it's very challenging to try to support them. The solution sometimes is to link them to countries that are geographically close where we can direct the patients to access treatment.

For patients from complex countries who managed to move out: we have managed to provide them with some support. For example, in the case of Palestinians who have managed to escape to Egypt, we have been able to liaise with the Egyptian Haemophilia Society to ensure they have access to some of the products sent via the WFH Humanitarian Aid Programme.

Can you talk about some of the achievements/developments in haemophilia care and governance in this region?

One big achievement we have seen in some of the NMOs is a shift from being HCP led to being completely patient-led, but with a medical advisory committee that continues to provide them with the medical expertise that they need. This has happened through a lot of work with the NMO and excellent awareness on the part of the NMO themselves.

Another achievement is the NMOs that have developed so well that they have become the go-to resource for all things haemophilia in their countries and who have a solid relationship with the doctors and with the ministry of health.

In terms of treatment, countries that have been able to develop their own national treatment guidelines have been better able to advocate to the government for improved access to therapeutic products and prophylaxis.

What are a few challenges of your role?

One key challenge is that we can't have programmes in all of the countries in my region. Another massive challenge is not being able to visit some countries and engage with them on the ground. The only way that we can really learn how WFH can better support the countries is by seeing them in their own context and by bringing all the stakeholders around the same table together (government, medical professionals, patients). Unfortunately, there are a good number of countries (at least 5 or 6) that we cannot visit. We compensate for that by meeting them at the WFH Congress and at HEMNET meetings when they can travel to these events. We also offer capacity-building opportunities abroad (via the fellowships programme and other training opportunities).

Another big challenge is advocacy. We still have a lot to do in terms of strengthening the NMO leaders' capacities to advocate for prophylactic treatment and for more sustainable access to therapies.

Another issue is ensuring that NMOs have enough motivated volunteers that want to put in the work because they see the NMO as their own.

Does the fact that most NMOs are led by volunteers without staff constitute a challenge?

Yes, it can do, as NMOs are volunteer led who are very busy in their daily lives, whether they are healthcare professionals, individuals with bleeding disorder or their family members. The majority of NMOs in the WFH are fully volunteer-based organisations. If the NMOs could recruit staff, it would, of course, make their operations easier, but not everybody has that luxury. In this regard, the Irish Haemophilia Society is very well positioned.

What is your favourite part of your job?

In addition to what I said above, we are in a job where we deal mainly with volunteers. We also have many experts among the volunteer community, without whom we would not be able to be a global leading organisation in the field. A lot of people are motivated to make change for our global community. They are easy to work with because they already have the fire inside them. At the country level, what some of them are missing is the resources, and they will make strides fast with a clear agenda. These volunteers have been able to do wonders in their countries. This is like climbing the Himalayas; when we are able to support people to make changes, it is a huge reward . We are working closely with them all towards the WFH vision of "Treatment for All".

Thank you to Rana Saifi for taking part in this fascinating interview about her work.

My Gene The

I was treated with factor IX (FIX) gene therapy as part of a phase three chinical trial in February of 2020 some 54 months ago. At the time of treatment, I was 62 years old and had lived a life encompassing all of the generations of haemophilia treatment.

Until I was age 14, I had no access to any regular treatment and bleeding episodes went untreated. As a consequence I had damage to my left knee, right ankle and right elbow. At age 14 I had my first treatment with a factor concentrate which was actually a prothrombin complex concentrate containing a mixture of factor II (FII), factor VII (FVII), FIX and factor X (FX). For the next three years, if I required treatment, it was fresh frozen plasma which necessitated a 400 mile round trip from Killarney to the treatment centre in Dublin. Obviously given the time it took to get to Dublin, a bleeding episode in a joint would be well advanced by the time that the plasma was infused.

When I moved to Dublin to go to college at age 17 I had access to home treatment with prothrombin complex concentrate. This was the treatment available until my early 30s when I switched to plasma derived FIX concentrate. Then in my early 40s recombinant FIX became available and this was my treatment of choice until my late 50s when I switched to extended half-life FIX concentrate. I did not start prophylactic treatment until my mid 50s. Of all of these treatment changes, in my view, the availability of home treatment where you could treat a bleed quickly as soon as it had started was the single biggest game changer. The availability of extended half-life FIX was also a significant milestone which allowed me to take prophylaxis once every 10 days while maintaining a trough that kept me in the mild range.

My decision to take part in a gene therapy clinical trial was a carefully considered one. I had been following the science for many years. Together with our clinicians, I had been involved in encouraging several of the companies who were conducting gene therapy clinical trials to consider Ireland as one of their venues for the trials. Over the course of the past 10 years, we had several companies who came to Ireland to discuss their gene therapy clinical trials with the clinicians and patients. I organised group meetings for the Society usually for 10 to 20 people with haemophilia who had expressed some level of interest in gene therapy to come along and hear more about the trials. We also worked with the Irish regulator to make sure that the trials could take place in Ireland.

During the course of these meetings and following my reading and research into gene therapy, I became convinced that the benefit risk ratio for me personally of participating in a gene therapy clinical trial was positive. I was convinced by the science of the potential of the gene therapy. Before enrolling I was aware of the unknowns and uncertainties. I knew that there was no guarantee of durability. I knew there was no guarantee of achieving a particular factor expression. I was aware some people may not get a response to gene therapy and would not achieve any significant factor expression. I was aware of the potential requirement to take steroids in the event of transaminitis in order to prevent any potential loss of factor expression achieved. I was aware of the theoretical risk of cancer from insertional mutagenesis because of some integration of the infused DNA into my own DNA.

rapy Journey

Despite these uncertainties, I felt that participating in a gene therapy clinical trial was the correct decision for me. I discussed this with friends and colleagues - both clinical and haemophilia organisation leaders who had a range of opinions in relation to gene therapy. I discussed this with my family, but they were very clear that they trusted my judgement and knowledge. Within the parameters of the uncertainties in durability, factor expression and predictability, I identified for myself my preferred personal outcomes while at all times being aware that these may not be achieved. If these were not achieved I believed I was ready to accept whatever outcome I did achieve.

I was hoping for a factor expression in the range of 20% to 60%. My preference was for my factor expression not to be too high as I wanted to maintain some of the potential cardioprotective effect of a slightly lower factor level given my age. I hoped for a durability of at least 10 years. I hoped for a decrease in chronic pain in my damaged joints and I hoped for the ability to be more physically active and physically fit. Obviously if I achieved a significant FIX level, it would mean I could stop prophylaxis. This in turn would free up some of my time and allow me to have some mental freedom from dealing with my own haemophilia. I was fully aware that given my roles with the Irish Haemophilia Society, European Haemophilia Consortium and ongoing work with the World Federation of Hemophilia, I would not and could not achieve anything like a "haemophilia free mind", as my entire working life is consumed with haemophilia and bleeding disorders. I did hope for some freedom from dealing regularly with my own haemophilia including in areas such as not having to take prophylaxis on the days of long-haul flights and carefully planning any significant physical activities to coincide with prophylaxis days.

There were other factors in my decision also. At my age of 62, if it took several years for gene therapy to be licenced and reimbursed in Ireland, I may have missed my opportunity to receive it. I have lived my entire life with severe haemophilia - I thought it would be interesting to try life possibly without severe haemophilia. I also wanted to lead. Despite the meetings over several years, no person with haemophilia in Ireland had participated in a gene therapy clinical trial. I was the first and I was quickly followed by two others.

With the decision made I entered into a lead in period of approximately 6 months before the gene therapy was infused. During that time period I had to keep an electronic diary recording and including any bleeding episodes and prophylaxis. I also engaged extensively with the research team at the centre where the gene therapy would be infused.

In the year prior to my gene therapy in 2019, I had travelled abroad frequently to conferences and to deliver lectures and I travel extensively for work. I had several conversations with the research team in relation to ensuring that we could schedule my follow up appointments ideally allowing me to maintain some of my work commitments abroad. Having said that, I was fully aware of the monitoring and follow up requirements and fully committed to the protocol. I wanted the gene therapy to be successful and I did not want to jeopardise the potential for success or jeopardise the trial protocol because of travel commitments. The monitoring visits required a visit once weekly for the first 12 weeks, followed by monthly visits for the rest of year one and bi-annual visits after that up to the end of Year 5. I was fully committed to making all of these visits and willing not to travel if that would interfere with my ability to stick to the protocol.

Ironically, that was not necessary as two weeks after my dosing, the COVID pandemic struck and I had no travel commitments for at least the next 18 months. This made it easier to manage my diary and schedule all the monitoring visits while simultaneously making it more difficult as it meant I had to attend a hospital setting very regularly during a pandemic at a time when most people did everything possible to avoid going into any hospital. The research team made this easier by facilitating the visits in a non-clinical setting where potential exposure to Covid 19 was very limited.

The gene therapy infusion day was relatively simple and uneventful. I attended at the research facility near my haemophilia treatment centre with my wife about two hours prior to the infusion to have some blood tests, final checks and final conversations. I received the infusion over about a 90 minute period, waited two hours and went home. The procedure was simple although I was aware that they had an emergency team ready if required in case of adverse reaction. I was very relaxed during the day but that evening I was emotionally drained as I realised what a momentous day this had been for me. I then had weekly visits for the first 12 weeks and indeed I exceeded this for several weeks by having twice

weekly visits. I wanted to make absolutely sure that there was no possibility they would miss any increase in liver enzymes which could result in loss of expression if there was a delay in diagnosing this and in commencing steroids. The visits were very well managed in a non-clinical space due to the COVID pandemic. The only adverse event I had was a decrease in my iron due to the sheer volume of bloods being taken on a weekly basis. In the first year I had twice weekly visits for the first three months and then monthly visits. In total in year one I visited the centre 30 times. I adhered fully to the protocol and abstained from alcohol for three months pre gene therapy and two years post gene therapy (exceeding the one year recommended.)

My outcomes from day one were good. My FIX level increased from week one and has stayed in the high mild or normal range over the past 54 months. Thankfully I did not require steroids as my liver enzymes never increased. I did have some decrease in chronic pain in some of my damaged joints partly I suspect due to gene therapy and partly due to additional time since my knee replacement in 2018. I was fitter and more active in the first year post gene therapy due to gene therapy and to the fact that I was not constantly travelling and was able to get into a balanced regime of exercise and diet. On the first anniversary of my gene therapy I was walking with my family in the Dublin mountains when I slipped and fell off a low wall onto some rocks. Despite this I did not get a bleed and this was a revelation to me. On another occasion in the first year I dropped a 2 kg dumbbell on my barefoot and again did not get a bleed. I have had two bleeding episodes since commencing the gene therapy, one of which was spontaneous and one which was due to a trauma. I have also required FIX on a couple of occasions to top up my factor level prior to a minor surgery or procedure.

Now that we're in a post COVID environment I am travelling again for work. I generally do not bring any FIX with me unless it is a long trip or a trip to a developing or emerging country where I would have difficulty accessing treatment if required. For exercise I walk routinely. I commenced using alcohol moderately two years after my gene therapy infusion. I had a brain lesion last December which required lengthy surgery. Thankfully, the lesion was benign. It was checked to see if it was in any way related to my having had gene therapy. While the final results are not yet available, preliminary findings and the type of lesion were both strong indications that it was not linked to gene therapy and may in fact have been growing slowly for several years. I am now also dealing with some other minor health issues not related to haemophilia.

I continue to follow the science around gene therapy very closely and I have to say that personally I have no treatment remorse. I made the correct decision for me at the time. My outcome has been good, but I would like to think that even if it had been less satisfactory, I would not have treatment remorse because I had fully thought through all the outcomes that may occur and I had managed my expectations accordingly. The key is to make a fully informed decision for yourself based on information, discussion and careful consideration of all the potential benefits and risks with input from the clinical team, family and also from the Society.

Brian O'Mahaony, Chief Executive

Upcoming Events

2024

16 OCTOBER	Medical webinars: Women & Bleeding Disorders
18 - 20 OCTOBER	October Members' Conference
30 OCTOBER	Medical webinars: Update on Novel Therapies
20 NOVEMBER	Medical webinars: Update on von Willebrand Disease
27 NOVEMBER	Medical webinars: Future of Paediatric Care for Children with Inherited Bleeding Disorders:

2025

7 - 9 MARCH	Annual General Meeting & Conference
17 - 19 OCTOBER	October Members' Conference

News from the Comprehensive Care Centres

Memo from the National Coagulation Centre on supply shortage of NovoSeven®

(Date 20/08/2024)

What is the supply issue with NovoSeven®?

There is a global shortage of NovoSeven® stocks due to a supply chain issue. The company, Novo Nordisk, are working to resolve this issue as quickly as possible. However, the likelihood is that Ireland will experience shortages in supply of NovoSeven® which will last until at least the end of the year.

In the meantime, the treatment centres are working closely with the National CFC coordinator Ms. Evelyn Singleton and with Novo Nordisk to manage the stocks we have and conserve them for acute bleeding or urgent procedures as well as maintaining stocks for the small number of patients who use NovoSeven® for prophylaxis.

Who is affected?

Only people who have NovoSeven® as a treatment of choice are affected by this shortage. Within this group, many people have other treatment options instead of NovoSeven®. Please talk to your treatment centre if you would like to learn more about your treatment options.

Will I be able to have treatment for acute bleeding or urgent surgery?

We will be able to treat acute bleeding and urgent surgery with NovoSeven® if you need it.

Will there be any change to planned surgeries or procedures?

We will defer any non-urgent procedures where NovoSeven® is needed to prevent bleeding. If it is possible to use a different treatment to prevent bleeding after a planned procedure, then your treatment centre will discuss this with you.

What will happen for people on prophylaxis with NovoSeven®?

Prophylaxis with NovoSeven® will continue as usual.

Will there be more information provided as the situation changes?

Yes, there will be updates sent to the Irish Haemophilia Society as soon as available from the Novo Nordisk, the Irish treatment centres and the international patient organisations.

Notice to Patients on Home Treatment using the the Mpro5HX (Home Treatment App)

As you may be aware, we had recent reports of users not being able to log in or scan their CFC barcodes on the home treatment app (Mpro5Hx). We had reported this to the company who manage the home treatment app and they have identified the cause of the problem.

The reason you are unable to scan is that some users have had updates to their IOS and Google software on their phones. The security within the new update is not compatible with the barcode scanner within the Mpro5Hx app. Those who have not yet updated to the latest software update from IOS and Google appear to be unaffected and continue to be able to use the Mpro5hx app.

The company have informed us that the only solution is that we need to release the new version of the home treatment app which we have been working on for the last while. This will resolve the issue for those who are currently unable to scan and for those who will need to update to the latest software in the near future.

We are working closely with the company who designed the app and we hope that the app will be available for you to use in the next two weeks. We will be back in contact once we know the release date for the new home treatment app.

In the meantime, you can continue to use your medication. Please keep the empty medication boxes so you can scan them once the issue has been resolved. You should document the date that you gave yourself the treatment on the empty boxes and the reason you gave the medication.

We apologise again for any inconvenience this may have caused & thank you for your patience. If you have any queries regarding your home scanning please contact, the haemophilia nurses in your treatment centre.

Staffing Update

We are delighted to introduce Lena Byrne as our newest member of staff. Lena joined the office in August 2024.

Lena's role is that of receptionist/administrative assistant.

Lena is the first point of contact through reception and fields all incoming calls and emails. Lena also offers general administrative support, assists with the organising of events and meetings, assists with the day-to-day running of the office and manages the apartment bookings.

We are thrilled to have Lena with us and are looking forward to continuing to work together!

Strategic Planning Update

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 are sending out a survey to members digitally. We are planning to send this out to members in the near future. Members will have two weeks to fill in the survey. We are hoping to get a good response from many different cohorts of members, including those with von Willebrand disorder, rare bleeding disorders, youth, volunteers and women with bleeding disorders.

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.

Register by calling the office on 01 657 9900.

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