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National Haemophilia

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WFH 2024 Congress

Reports from Australian delegates

World Haemophilia Day 2024

Celebrating around Australia

Hepatitis C

An update

HAEMOPHILIA FOUNDATION AUSTRALIA

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Cover: Madrid streetscape
Photo: HFA

New AHCDO ABDR Research Fellow

The Australian Haemophilia Centre Directors' Organisation (AHCDO) is pleased to welcome Ashley Fletcher as the new Australian Bleeding Disorders Registry (ABDR) Research Fellow. Ashley brings over 20 years of expertise in epidemiology, clinical data management, and project leadership. In his new role, he will oversee data collection and analysis, oversee the ABDR Data Managers Group, and spearhead various research initiatives, significantly advancing AHCDO's objectives.

Previously, Ashley served as a Senior Research Fellow at Monash University, managing projects for the Australian and New Zealand Society of Cardiac and Thoracic Surgeons (ANZSCTS) Database. His responsibilities included project planning, stakeholder collaboration, and policy development. At BioGrid Australia, he coordinated investigator-led research projects and supported clinical research and software development.

Ashley's career also spans roles at Cabrini Health, managing the Australian Rheumatology Association Database (ARAD), and at Cancer Council Victoria, leading geographic analysis for cancer services. He has contributed to genetic services and public health genetics at the University of Melbourne, Murdoch Childrens Research Institute, and the Australian and New Zealand Intensive Care Society (ANZICS).

An active member of the Australasian Epidemiological Association, Ashley holds a Master of Epidemiology from the University of Melbourne. He enjoys sports, outdoor activities, web design, walking his 2-year-old German Shepherd and eighties music, and has published over 30 papers in refereed journals.

Gavin Finkelstein

President, Haemophilia Foundation Australia

From the President

WFH WORLD CONGRESS

The WFH World Congress brings the worldwide community together, which I think is enormously important. I was privileged to attend the 2024 WFH Congress in Madrid. It was exciting to hear updates on what's happening worldwide - with women, clinical trials, gene therapy, you name it. As you will read in this issue of *National Haemophilia*, Australia was well-represented at Congress and our speakers and delegates acquitted themselves well.

With floods in Dubai, my flight was delayed by a couple of days and I was disappointed to miss the Global National Member Organisation (GNMO) Training workshop that was held before Congress. The plan had been for me to present the HFA poster about the Bleeding Disorders Roundtable and then join a panel to discuss collaborating on future directions. Fortunately, the poster made it to the Training, even if I didn't. It described how HFA is bringing together representatives from HFA and all the health professional groups for roundtable discussions: the Australian Haemophilia Centre Directors' Organisation (AHCDO), and the nursing, physiotherapy, psychosocial worker and data manager groups. The Roundtable meets three times a year to discuss current issues from the perspective of each discipline and has been a great opportunity to collaborate.

Together with Natashia Coco, HFA Executive Director, I also attended the WFH Cornerstone Initiative lunch during Congress. The Cornerstone Initiative aims to close the gap in treatment by providing support, expertise, and training to countries with minimal levels of care. The Initiative is currently active in Tanzania, Mozambique, Benin, Mauritania, Madagascar and Tajikistan. It was great to hear stories form these countries and how the program is assisting so many in the bleeding disorders community. HFA has supported this program financially.

World Haemophilia Day is one way of supporting WFH initiatives like these. Thank you to everyone who supported World Haemophilia Day – we had over 80 locations turn red to raise awareness.

RESEARCH

To make sure people with bleeding disorders have a voice in their future treatment and care, HFA regularly promotes relevant research studies. With several new therapies currently going through the process to become available in Australia, you may have noticed that you are being invited to participate in quite a few. HFA will also launch a more detailed study on treatment goals and preferences in haemophilia in a few months' time. I would encourage you to take the opportunity to take part in some of these studies, if possible, and give your opinions and insights.

HEPATITIS C

The release of the report from the UK Infected Blood Inquiry has raised questions in the Australian community about HFA advocacy relating to hepatitis C. We have outlined the continuing work on our hepatitis C strategy in the article about the UK Inquiry and Australia in this issue of *National Haemophilia*. We understand that revisiting these experiences may be an upsetting time for some people in our community. Please reach out for support if this is the case for you – suggestions for support are at the end of the article.

UK Infected Blood Inquiry and Australia

The UK government Infected Blood Inquiry investigating contaminated blood and blood products released its report on 20 May 2024 after examining the evidence for 7 years. The UK government announced a financial compensation scheme to 'victims of infected blood' the following day. You can read the report at https://www.infectedbloodinquiry.org.uk/reports/inquiry-report.

Some of our community members have been asking what this means for Australia.

THE AUSTRALIAN SITUATION

In Australia the initial situation with collecting blood donations and testing the blood supply was different to the UK. Australia was one of the first countries in the world to introduce hep C testing of the blood supply and had a policy of self-sufficiency in the blood supply (ie, sourcing donations from Australia only). There was an Australian Senate Inquiry into **Hepatitis C and the Blood Supply** in 2004, which investigated and reported on the Australian situation and an Australian government **Inquiry into Hepatitis C in Australia** in 2015.

WHAT HAS HFA BEEN DOING?

In 2006-9 HFA undertook a hepatitis C needs assessment and described the burden of hepatitis C on our community members in our **Double Whammy** (2007) and **Getting it Right** (2009) reports. The HFA 2020 **Getting Older needs assessment** found that, although the greater majority of surviving people with bleeding disorders who had acquired hepatitis C have now been cured, many have ongoing health, financial and support issues. HFA has a committee working on a hepatitis C strategy to address this, including the approach to HFA's advocacy, which has been ongoing for more than 20 years now.

Eliminating hep C

In the HFA *Double Whammy* report people with bleeding disorders and hep C underlined that their highest priority was a cure. When the new treatments became available in Australia in 2016, we focussed on reaching as many affected people as possible to promote access to treatment and a cure. We are grateful to the HTCs for their tireless efforts to review their patients and encourage them to have hep C treatment and be cured.

Financial schemes

Although the 2004 Senate Inquiry into Hepatitis C and the Blood Supply recommended case management and financial support for health and community care costs of those who acquired hepatitis C through the blood supply, a formal program was never implemented.

Australian governments contributed to Hepatitis C Virus (HCV) litigation settlement schemes for eligible people who contracted HCV via the blood supply in Australia between 1985 and 1991, prior to the introduction of reliable screening tests for hepatitis C virus. However, eligibility for the scheme involved being able to link the individual's source of infection to a single donor with hepatitis C. HFA believes that nearly all people with bleeding disorders were excluded from eligibility as they had many treatments during this period and usually with clotting factor concentrates manufactured from the pooled plasma from thousands of donations and they could not identify a particular treatment batch or a single donor. Apart from a small ex-gratia payment that was made by the ACT government to people with bleeding disorders who acquired hepatitis C through the blood supply, there have been no other government financial recompense schemes for people with bleeding disorders in Australia.

HFA has been active over many years in hep C advocacy. Some key activities are below.

For a full list of HFA activities and links to the documents, visit the HFA hepatitis C strategy page on our website – https://tinyurl.com/hfa-hepc-strategy.

Overview of key HFA activities

2019-ongoing	HFA consultation with the bleeding disorders community and expert health professionals about ongoing issues with hepatitis C – see HFA *Getting Older with a bleeding disorder report* (2020). HFA is now working through a strategy to implement the report recommendations (government acknowledgement; health, financial and support issues).
2016-ongoing	HFA campaign to eliminate hep C in the bleeding disorders community and to promote liver health monitoring for those with cirrhosis. Includes: • work with Haemophilia Treatment Centres, hepatitis specialists and the community to overcome barriers to treatment and monitoring • education for the community and to GPs to reach those with mild bleeding disorders and those who are disconnected from HTCs.
2014-16	Community consultation on hep C treatment. Submissions to the Australian Government/ PBAC on access to new DAA hep C treatments
2015	HFA witness statements and submission to Australian Government **Inquiry into Hepatitis C in Australia**.
2010-ongoing	Further community consultation about financial issues. Meetings and correspondence with Australian governments on no-fault financial assistance scheme for people with bleeding disorders and hepatitis C and issues relating to out-of-pocket health care costs.
2006-2009	Needs assessment of people with bleeding disorders and hepatitis C and evaluation: **Double Whammy** and **Getting it Right** reports
2004	HFA Submission to Senate **Inquiry into Hepatitis C and the Blood Supply**, including a proposal for financial recompense
2003-4	HFA national campaign for universal access to recombinant clotting factor treatments (synthetic and virus-free)

The HFA Getting Older report highlighted that hepatitis C leaves an ongoing legacy for many in our community. We continue to work on a strategy to implement the report recommendations and will advise of any further steps and outcomes.

We are aware that revisiting these issues can be painful for some members of our community. We encourage you to reach out for support if this experience is challenging for you – eg, to your HTC social worker/psychologist or your preferred counsellor. You may also wish to seek a counsellor through your GP or contact a service like Lifeline (call 13 11 14).

World Hepatitis Day 2024

In July 2024 Australian landmarks will be glowing green to raise awareness about eliminating viral hepatitis. World Hepatitis Day is marked internationally on 28 July and is one of the World Health Organization's nine official global public health days. Green is used by the global NOhep movement – the colour of life, vitality and progress.

On World Hepatitis Day the international community comes together to step up efforts to eliminate viral hepatitis, in particular hepatitis B and hepatitis C. In 2024 the national theme is **It's time for action**.

Many people don't know that they have hep C. For example, both men and women could be at risk if they have a bleeding disorder and ever had a blood product before 1993.

Some people have been cured but still need follow-up for their liver health, especially if they have cirrhosis.

Good liver health is a hot topic for everyone in the bleeding disorders community - and in gene therapy good liver health is a requirement. We will be looking at strategies to manage your liver health as part of our World Hepatitis Day activities.

On World Hepatitis Day we are reminded to take action – know your hep C status, have treatment to cure hep C, where possible, and follow up on your liver health after treatment.

By talking to our friends, family or a doctor about testing, treatment, liver health checks and managing liver health we can work towards these goals.

It's also important to be aware that sadly, some people with bleeding disorders and hep C have advanced liver disease caused by long term infection. Close liaison between liver specialists and Haemophilia Treatment Centres is key to care and treatment. Research into management of advanced liver disease is ongoing.

Look out for more information and activities on our website and social media in the week leading up to 28 July.

FIND OUT MORE

Australian World Hepatitis Day website - www.worldhepatitisday.org.au

HFA World Hepatitis Day page - www.haemophilia.org.au/world-hep-day

Gene therapy webinar

Gene Therapy: 'Ask Me Anything' with Dr Michiel Coppens

Watch the interview here (transcript also available):

https://tinyurl.com/genetherapy-interview

In May 2024 we were fortunate to have Dr Michiel Coppens join us at the HFA office for an interview about gene therapy. Dr Coppens is a leading haematologist at Amsterdam University Medical Centers, The Netherlands, recognised internationally for his expertise in gene therapy.

Chaired by Dr Stephanie P'ng, Chair of the Australian Haemophilia Treatment Centre Director's Organisation (AHCDO), and led by questions from the community, Dr Michiel Coppens explained what gene therapy is, how it works, and how it can be used to treat people with haemophilia.

The interview opened with a thorough introduction to gene therapy, specifically AAV gene therapy, and dove into the results of haemophilia A and haemophilia B clinical trials. Dr Coppens' extensive knowledge of haemophilia and experience in gene therapy – he dosed the first Dutch patient with gene therapy back in 2015 – enabled him to provide information that is both detailed and easy to understand.

Dr Coppens then moved on to discuss the advantages – and disadvantages – of gene therapy. His advice for those considering this treatment is to consider who they are as a person, recognising that some people may be keen to try it, while others will prefer to wait and see.

The interview concluded with a Q&A session where Dr Coppens answered questions from our community. Questions ranged from eligibility criteria – will gene therapy be available to people with mild haemophilia? – to whether it could be used to treat type 3 VWD.

We want to thank Dr Coppens and Dr P'ng for taking the time to share their knowledge and experience. The full interview and a transcript can be found on the HFA website.

Research studies: why participate?

Image: Cottonbro for Pexels.com

You may have noticed that you are being invited to participate in quite a few research studies at the moment.

One reason for this is that there are several new treatments for bleeding disorders currently going through the process to become available in Australia. As part of this process there are studies underway to understand:

- the impact on people with bleeding disorders
- patient preferences
- to prepare the resources to support new treatment products – this includes 'Australianising' the language for questionnaires as well as support products and services
- how to improve treatment and care.

There are also other studies to understand the impact of a bleeding disorder, for example, on joints and muscles, where the aim is to improve health outcomes.

It can be hard to find the time when you are busy, but this is your opportunity to have a say about your experience and what you would like - now and in the future.

In Australia decisions about health services, treatment and care are strongly influenced by research in the area.

HFA will draw your attention to some research studies via e-news and social media as they become available.

We strongly encourage you to look at them and consider if you would be able to participate.

WHERE TO FIND CURRENT STUDIES?

HFA has a standard process to review research studies before promoting them.

Links to reviewed studies are in the **PARTICIPATING IN RESEARCH** section on the HFA website - https://tinyurl.com/HFA-research-participating .

There are three different categories of research:

- **Clinical research** – studies testing the impact of health conditions on the body and treatments, how well they work and how safe they are.
- **Market research** – studies evaluating people's preferences and experiences of treatment and care, products and services.
- **Social research** – studies about people's experiences to better understand how to improve their health and wellbeing.

HFA also occasionally conducts our own community surveys and research studies, such as the PROBE Australia Study. These are to help with representing people with bleeding disorders in Australia and with providing relevant education and programs.

You will find information about HFA studies under **RESEARCH** or in the **NEWS** items on the HFA website (www.haemophilia.org.au)

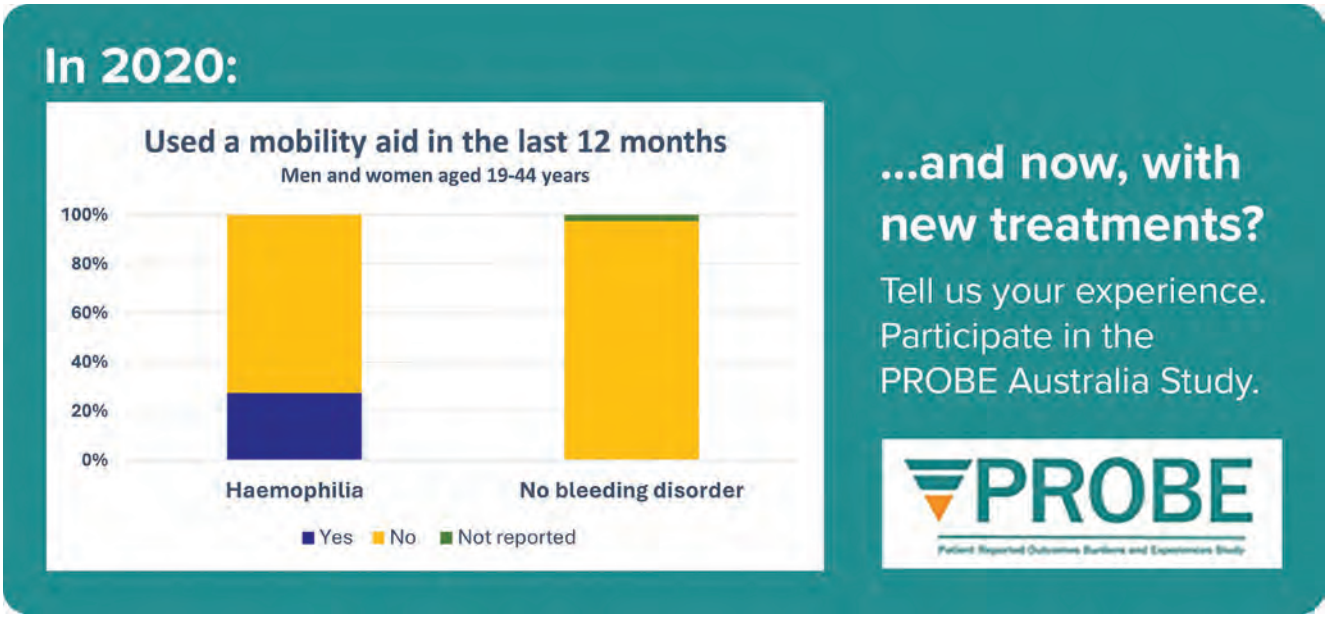
Any questions? Contact Suzanne O'Callaghan, HFA Policy Research and Education Manager on socallaghan@haemophilia.org.au or P: 1800 807 173.

Have you done the PROBE survey yet?

What difference have new treatments made to the experience of haemophilia in Australia?

The **PROBE Australia Study** helps HFA collect the evidence to advocate for new haemophilia treatments. And you can contribute to that evidence.

You can see from our 2020 PROBE study results how many people with haemophilia used a mobility aid like crutches back then – nearly 30% of men and women aged 19-44 years, compared to none of the people without a bleeding disorder in the same age group.



Please consider completing the PROBE survey and tell us what your experience is now.

We currently have about 250 responses. **For strong results we need about 150 more men and women to complete the survey.**

Who can take part?

- Men and women with haemophilia or who carry the gene
- AND men and women without a bleeding disorder, like family and friends, health professionals, and other people interested in haemophilia

Participate in the PROBE Australia Study today and contribute to our real-world evidence on haemophilia!

FIND OUT MORE

www.haemophilia.org.au/probe-australia-study.

WORLD HEMOPHILIA DAY
17 APRIL 2024
#WHD2024

Every year on 17 April World Haemophilia Day is recognised worldwide to increase awareness of haemophilia, von Willebrand disease and other inherited bleeding disorders. This is a critical effort since with increased awareness comes better diagnosis and access to care for the millions who remain without treatment.

It was spectacular seeing landmarks across Australia - and the world - light up red in support of bleeding disorders. Australia had over 80 locations turn red in support of the day.

In 2024 the international theme was **Equitable access for all: recognizing all bleeding disorders**. The World Federation of Hemophilia (WFH) vision of **Treatment for All** is for a world where all people with inherited bleeding disorders have access to care, regardless of their type of bleeding disorder, gender, age, or where they live.

Did you know, WFH estimates that over 75% of people living with haemophilia worldwide have not yet been identified and diagnosed.

WORLD HAEMOPHILIA DAY IN PICTURES

John Hunter Children's Hospital HTC team, Newcastle, NSW

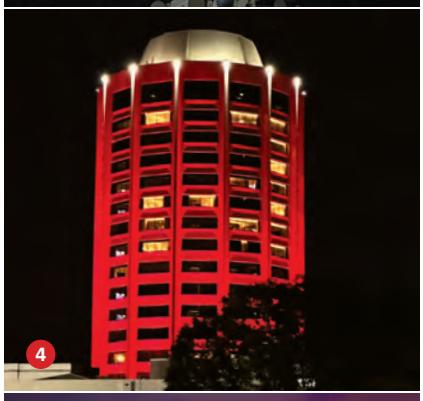
HFQ climbing the Story Bridge, Brisbane

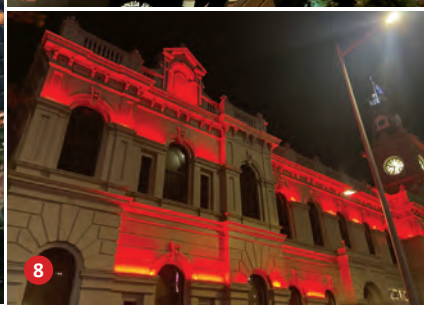
Goulburn Valley Library, VIC

Jet and Ollie celebrate in red, VIC

We thank everyone who participated in the day and the many landmarks that lit up red across Australia.

LIGHTING IT UP RED ACROSS AUSTRALIA





1. Telstra Tower, Canberra ACT
2. Wardandi Boodja sculpture, Bunbury WA
3. Customs House, Brisbane QLD
4. Wrest Point Casino, Hobart TAS
5. Bell Tower, Perth WA
6. The Glasshouse, Port Macquarie NSW
7. Story Bridge, Brisbane QLD
8. Drum Theatre, Dandenong VIC

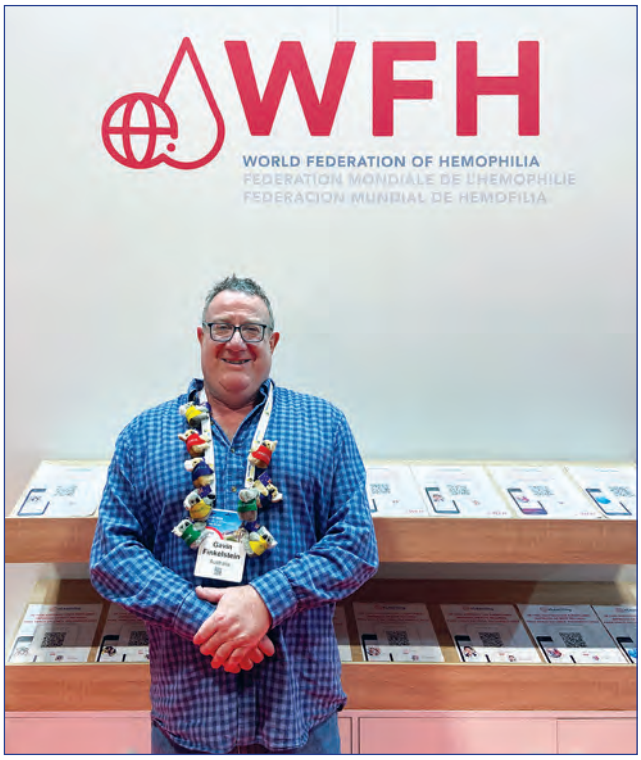
WFH 2024 World Congress

Australian Congress delegates

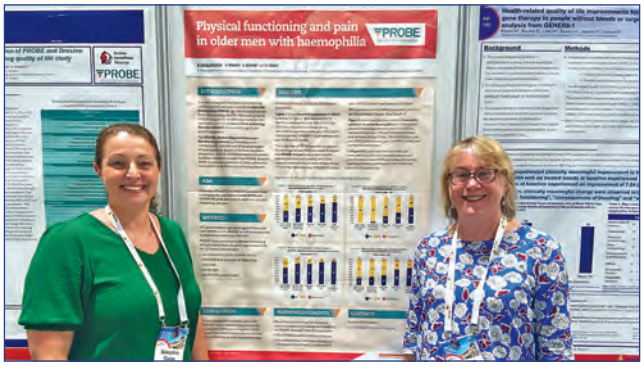
The World Federation of Hemophilia **WFH 2024 World Congress** took place in Madrid, Spain on 21-24 April 2024. It brought together more than 3,000 delegates from 135 countries – people with bleeding disorders as well as national member organisations like HFA, health professionals and industry. Congress is an important opportunity to discuss the latest information, debate some of the issues relevant to bleeding disorders and look to the future and was the first fully in-person event for the WFH since the WFH 2018 World Congress in Glasgow, Scotland.

Australia was well-represented at the WFH Congress as speakers, poster presenters and as delegates. In this issue of *National Haemophilia* we hear reports from delegates about some of the sessions they attended and what messages they took home from the discussions.

The 2024 WFH World Congress in Madrid and the EAHAD Congress in Frankfurt were attended by a number of specialist Haemophilia Nurses from Australia. The opportunity for nurses to attend the two Congresses came from a number of funding sources. This included funding from Haemophilia Foundation Australia and the Pfizer Nurse Education Award, as well as funding to three nurses from Sanofi; and one nurse self-funded. This funding enabled Australian Nurses to attend the Congresses to learn more about new advances in treatment and care and to bring this back to their local clinical settings. The impact this has on practice change to benefit patients and their families along with the networking opportunities is invaluable.



Gavin Finkelstein at the WFH stand



Natashia Coco and Suzanne O'Callaghan with the HFA PROBE poster

Suzanne O'Callaghan moderating the education strategies poster session with the French and Canadian presenters

Photos: HFA

CONGRESS REFLECTIONS

Australian delegates reflected on their experience at Congress.

This was my first time attending the World Federation of Hemophilia Congress in the beautiful city of Madrid, Spain. It was a truly amazing experience being able to learn and collaborate with the world experts in haemophilia. After attending several different health conferences within my career, it was very inspiring to see the number of people with bleeding disorders involved in the Congress and their input in the future direction of haemophilia management.

Bianca Da Silva, *paediatric physiotherapist, John Hunter Children's Hospital, Newcastle NSW*

The World Federation of Haemophilia Congress 2024 kicked off in Madrid, Spain. What a place to get together with people who are interested in the space of haemophilia! Team Australia did us proud with amazing presentations from HFA, nurses and physiotherapists.

Yuhsuan Lin (Yoshi), *haemophilia nurse, Royal Adelaide Hospital, SA*

Congress was such a wonderful opportunity to meet up with my international colleagues face to face, discuss all of the new developments being presented by the experts and come up with some great ways to go forward. And we were all so excited to be with each other in Madrid – it really added some happy energy to the meeting!

Suzanne O'Callaghan, *Policy Research and Education Manager, HFA, Melbourne, VIC.*

Representing HFA globally

Natashia Coco

GENERAL ASSEMBLY

The World Federation of Hemophilia (WFH) Global Assembly is the annual meeting for all National Member Organisations (NMOs) to come together. The meeting was an insight into the international work of WFH and showcases issues for both developing and developed countries. It was an honour for me to represent Australia.

I voted on behalf of Australia on WFH committee member positions, and the destination for WFH Congress in 2028 – which is Chicago.

At the meeting Benin and Rwanda became full NMOs; and Burundi, The Democratic Republic of Congo, Gabon, Guinea, and Sierra Leone became associate NMOs. This brings the total number of NMOs in the WFH family to 152.

CONGRESS

WFH Congress is an experience that is information overload, but it's very meaningful at the same time. It was great to meet others and see people we have known for a long time from around the world and hear all their stories, the successes and the failures. How can we do better?

Personally, I left Congress feeling so excited about what is potentially around the corner with treatment for our community, but also thinking about how we are going to educate our community to support the treatment decision-making process, and how we can look at other countries' learnings to move forward in the best way.

Congress this year had over 3,000 delegates from 135 countries. With an extensive and full program over three days it was wonderful to hear experiences from around the world and the personal stories. I really focused my session attendance on gene therapy and emerging treatments and women. The sessions I attended answered most of my questions and I ended Congress feeling much better informed.

On the last evening HFA hosted an Australian get together so that all our delegates could catch up and chat about what we have got out of Congress. Everyone was elevated, tired and so excited. In total we had 20 Australians in Congress (supported financially in different ways). You will read some of their reflections and reports in *National Haemophilia*.

In 2026 WFH Congress will be hosted in Malaysia, closer to our shores.

Natashia Coco is HFA Executive Director

Congress – a Foundation perspective

Haemophilia Foundation Australia President Gavin Finkelstein spoke with HFA about his experiences at the WFH 2024 World Congress.

Natashia Coco, HFA Executive Director, and Gavin Finkelstein

What was important to you about Congress overall?

It's always important to get the worldwide community together.

It's good to see what is happening in other countries who are similar to the way we operate in Australia, like the UK, Canada and Scandinavian countries. Are they doing anything innovative that is working that we can try to replicate here? How are they managing the range of new treatment products that are coming, and making them available to their patient group? How is shared decision-making working for them – are they using the WFH Shared Decision Making tool or other alternatives?

I enjoyed catching up with my international colleagues. When you look at it, their issues are very similar to what we are experiencing here. There's a range of new treatment products coming to market that may have benefits for different members of the community. Making sure choice is available to both treaters and patients is a common concern – to choose the product that will provide the best outcome and experience for an individual. In some cases, it might be they feel more comfortable with device they use to administer it, or that it is sub-cutaneous delivery rather than intravenous; or the length of time between administration and increasing the trough level with their factor levels. These days we are seeing a smoothing of the trough level line, which is pretty exciting.

Congress is also an update on what's happening worldwide: with women, clinical trials, gene therapy, you name it.

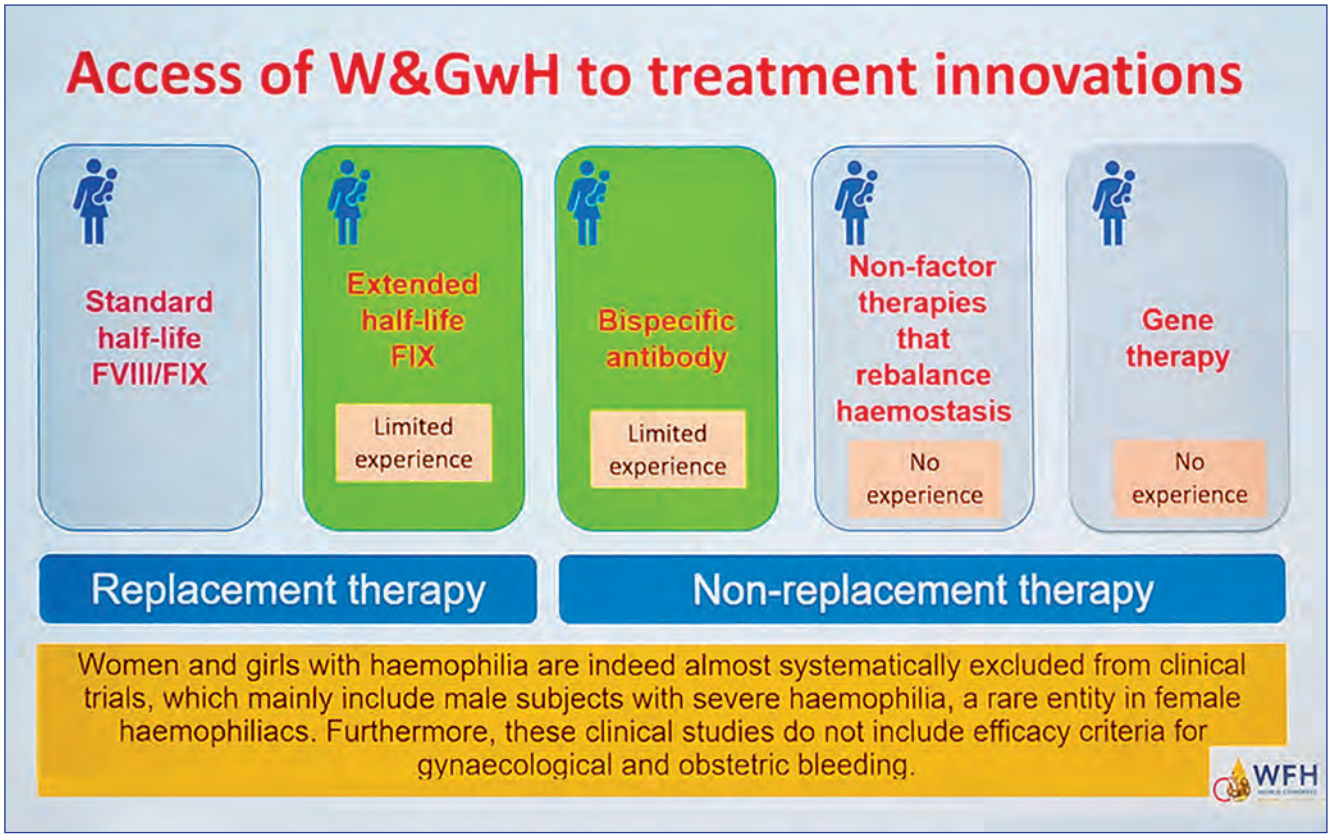
Which sessions stood out for you?

The women's sessions were a highlight. There is still a way to go. Things are moving quickly, but not quickly enough, as the women said in the sessions.

There were two aspects that stood out for me.

Clinical trials need to include women, including gene therapy clinical trials. It's an inequitable situation if women are excluded and this is long overdue. In the past including women has been seen as too complicated, but now researchers are seeing the benefits and, I believe, just need to get their parameters and doses correct.

There are women with severe forms of a range of bleeding disorders, including haemophilia, and there are women who are appropriate for prophylaxis and emerging therapies and need to be considered. This needs to be a primary thought when developing clinical trials, rather than



secondary, and it will help to avoid any issues in the future. It's a big change in the way research into treatment is looked at, but an important one.

Gene therapy was another issue where there is still a way to go. I don't know that a large proportion of the community will want to access gene therapy at the moment, so it is important that the efficacy of gene therapy does improve over time. Opportunities for further doses in the future are also crucial. We need to take into account that some people are not eligible because of AAV immunity and that gene therapy fails for some people. To be realistic, in the next few years many people will still choose to use factor and non-factor treatments rather than taking on gene therapy.

What was your take home message from Congress?

While it has been exciting to see the progress with gene therapy, it is important to recognise the range of very good therapies becoming available to people with haemophilia.

It was also the first time I had heard of women being included in clinical trials and that was a really positive message. Women are a big part of the affected community. Research and development at pharmaceutical companies will want to take this seriously, maybe with different arms of clinical trials to test parameters, doses etc.

More imaginative approaches to treatment for von Willebrand disease (VWD) was also clearly on the agenda, including alternatives to plasma-derived products. For example, I was interested to hear about existing products for people with factor VIII (8) deficiency being used to treat VWD. There do need to be more treatment options for people with type 3 VWD on prophylaxis, particularly.

On the world platform, we are always happy to be involved with our regional neighbours, but Congress highlighted how valuable it is to connect to countries with similar health systems as well, both around treatment and around managing the challenges of Foundation development. For example, succession planning and dealing with treatment advocacy into the future are common issues – and how you encourage younger community members to understand the importance of being involved with their local Foundation. It was great to talk with my international NMO colleagues and I'm looking forward to our ongoing discussions.

Pain in children with bleeding disorders

Daisy Regan

At the WFH 2024 World Congress in Madrid, I attended a session named 'Pain and PWBD in the new era: Understanding pain beyond joint damage'. This session had speakers from a diverse group of specialties including medical specialists, scientists, physiotherapists, dentists, psychologists and researchers who all contributed to the discussion, and outlined and provided insight into multidisciplinary approaches, educational strategies, and coping techniques to address pain adequately in patients with bleeding disorders.

Pain and PWBD in the new era: Understanding pain beyond joint damage

Chair ~ *Kate Khair, UK*

Concept of pain, IASP concept, adapted to BD - active role of patient in the control and knowledge of pain
~ *Roberto Ucero-Lozano, Spain*

Physiotherapy approach to pain in BD - exercise/ movement
~ *Cameron Cramey, Australia*

Pediatric perspective on pain
~ *Adriana Linares, Colombia*

The reality of living with chronic oral and oro-facial pain ~ *Rebecca S. Schaffer, USA*

Psychosocial perspective on pain management - techniques to use
~ *Gaby Golan, Israel*

I found the speaker Adriana Linares' session particularly interesting and thought provoking due to the focus on the paediatric and specifically the 'paediatric perspective on pain'. Dr Linares is a Scientific Director and bioethicist based in Bogota, Colombia who presented on why health professionals are statistically less likely to ask paediatric patients if they are experiencing pain at clinic reviews, compared to the adult cohort.

With pain being one of the main clinical characteristics of haemophilia, it was very interesting to see the responses around the room during the session when healthcare workers were asked if they routinely ask their paediatric patients if they have pain specifically, separately to asking if they have experienced any bleeds since the previous review. Few people indicated that they do ask routinely. Comparatively, the patients with haemophilia and other bleeding disorders who were present were invited to raise their hand if their team asks them if they have pain at appointments. There were very few hands raised to indicate that patients are asked routinely.

For myself as a paediatric haemophilia specialist nurse, I could honestly say that I am very proficient in asking patients and their families if there have been 'any bleeds or concerns' recently; however, it was brought to my attention that the question that should be included is 'do you have any pain?' as it is far more open-ended and the child is invited to disclose more information.

This has been a very helpful reflective process for me, particularly as a nurse new to the specialty of haemophilia (less than 12 months specialising), and has sparked a new consideration in regards to expanding questions I would ask paediatric patients at their clinic appointments.

Australian haemophilia nurses were assisted to attend WFH World Congress through funding from a variety of sources. For more information, see page 12.

Image: Tahla Riaz for Pexels.com

Daisy Regan is the Haemophilia Nurse Consultant at the Michael Rice Centre for Haematology and Oncology, Women's and Children's Hospital, North Adelaide, South Australia

Ultrasound, bleeds, joints and sport

Bianca Da Silva

Musculoskeletal workshop 1: In-depth joint ultrasound imaging: Hands-on workshop

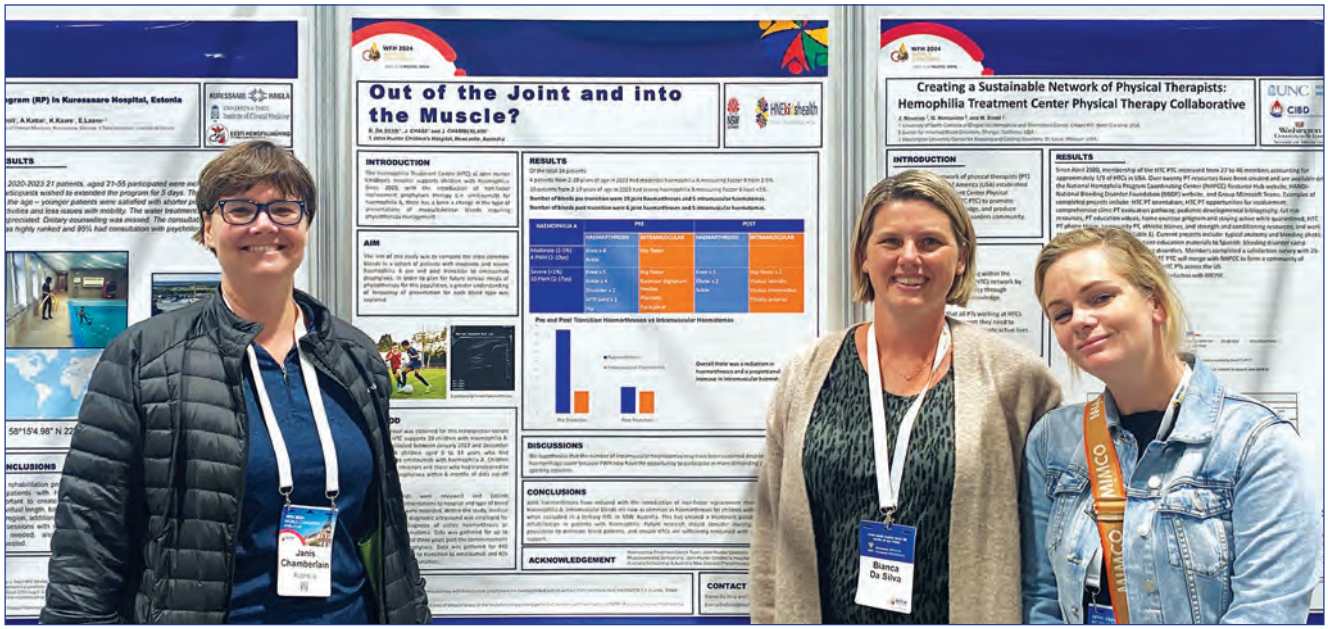
Moderator ~ *Cindy Bailey, USA*

Speakers ~ *Eric Chang, USA; Carlo Martinoli, Italy; Annette von Drygalski, USA*
And laboratory instructors and panellists

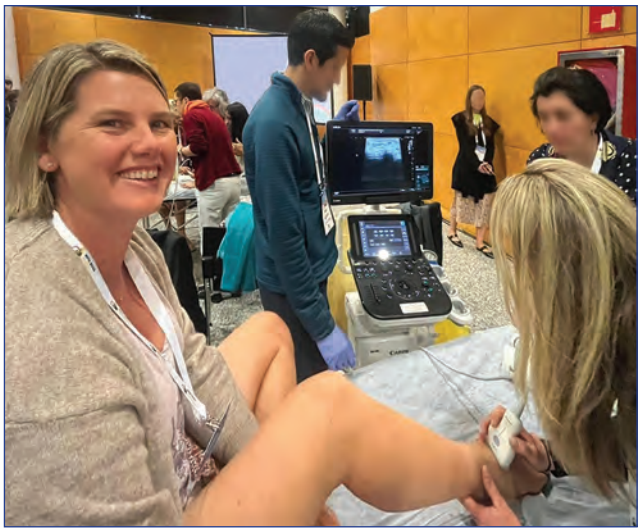
My first day at WFH 2024 World Congress consisted of a full day ultrasound workshop at the Professional Development Day, with the focus on teaching how to detect the presence or absence of a bleed (largely in the joint spaces) using point-of-care ultrasound (PoCUS), which is used routinely in some Haemophilia Treatment Centres in the United States of America.

PoCUS is done at the bedside (i.e., point of care) by using portable ultrasound equipment to support a clinical assessment, with the idea that pain can be used in early identification to detect the presence or absence of a bleed. It does not replace current imaging that Haemophilia Treatment Centres are currently using such as ultrasound, CT or MRI that are performed by radiologists. It was demonstrated that it can also be used in the clinical setting to monitor the progression of a bleed, and how to use doppler imaging to assist with clinical decision making in whether a person with a bleeding disorder can consider returning to sporting activity.

There was a particular focus on hands-on practice and teaching, with a number of skilled haematologists and physiotherapists/physical therapists teaching small groups how to use different musculoskeletal placements of the ultrasound transducer to identify bony and soft tissue landmarks, and how it would theoretically look in the presence of a bleed. There was also discussion about the results of a recent pilot study performed by the University of California San Diego, where patients were trained to be able to remotely perform joint self-images using a portable ultrasound device guided by a clinician utilising Telehealth. This demonstrates a possibility and future direction for the use of ultrasound in haemophilia care.



L-R: Dr Janis Chamberlain, Bianca Da Silva and Jaime Chaise with their poster, 'Out of the joint and into the muscle'



And then the Congress sessions began! There were different tracks/streams at the conference including medical, PWBD (people with bleeding disorders), MSK (musculoskeletal), dental, psychosocial, WGBD (women and girls with bleeding disorders), nurses and lab sciences. As a physiotherapist, I largely attended the musculoskeletal track, however there were a number of interesting sessions across the conference that regrettably I was unable to attend. This further reinforces the diversity of topics and different areas to consider in management of haemophilia as a person with a bleeding disorder or a clinician involved in patient care.

Musculoskeletal workshop 3: Optimizing musculoskeletal management for PWBD

Moderator ~ *Greig Blamey, Canada*

Speakers ~ *Adolfo Llinas, Colombia; Luigi Solimeno, Italy*

And discussion leaders

An additional highlight during the conference was the optimising musculoskeletal management for people with bleeding disorders session. During this session, attendees were able to work in small groups with a haematologist, orthopaedic surgeon and physiotherapist/physical therapist supporting each group as case studies were discussed, and then a greater discussion was conducted as a larger group. The ability to brainstorm and discuss cases with different clinicians from around the world was extremely valuable.

Game-changing health: Bridging new treatment, sports, and everyday living

Chair ~ *Cindy Bailey, USA*

Speakers ~ *Hazri Aris, Malaysia; Manuel Rodríguez López, Spain; Clive Smith, UK; Olaf Versloot, Netherlands*

The final session on game changing health: bridging new treatment, sports and everyday living was uplifting. It reinforced the importance of working closely with people with bleeding disorders to support them to achieve their desired goals. Hazri Aris and Clive Smith shared their experiences and physical endeavours including their challenges, and reflected on the future and changes to treatments and what this may mean for people with bleeding disorders.

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Aguero P, Barnes RFW, Flores A, von Drygalski A. Teleguidance for patient self-imaging of hemophilic joints using mobile ultrasound devices: a pilot study. Journal of Ultrasound in Medicine 2023 Mar; 42(3):701-712. https://doi.org/10.1002/jum.16084

Bianca Da Silva was assisted by funding from HFA and the Dr Ho MSK Scholarship, John Hunter Children's Hospital, to attend the WFH 2024 World Congress.

Photos: Bianca Da Silva

Bianca Da Silva is Head of Department, Paediatric Physiotherapy, John Hunter Children's Hospital, Newcastle, NSW.

Body art – tattoos and piercings

Jaime Chase

At the Nurses' Workshop at WFH 2024 World Congress, Scott gave a fascinating presentation about the patient experience of body art with severe haemophilia A and the planning you may need to take when you are contemplating it.

Nurses workshop: Beyond factor
Body art - tattoos, piercings
Speaker (patient perspective) ~ *Scott McLean, UK*

Body art is a diverse form of artistic expression that involves decorating or modifying the human body. It encompasses various techniques or styles, including tattoos and piercings.

Tattoos are perhaps the most well-known form of body art, where ink is injected into the skin to create permanent designs or images. These designs can range from intricate patterns to realistic portraits, and they often hold personal significance to the wearer.

Piercings involve puncturing the skin to insert jewellery, typically in areas like the ears, nose, eyebrows, lips, tongue, or belly button. Like tattoos, piercings can be a form of self-expression or cultural condition.

Scott spoke eloquently about his journey with his body art and how sometimes he needed to adjust his treatment regime and discuss a plan with his Haemophilia Treatment Centre (HTC) before commencing any body art activities. He also spoke about being honest with your proceduralist before commencing.

His humorous descriptions of when he was younger and deciding to get tattoos without treatment prior to the procedure were very descriptive of what could go wrong in this type of situation.

His final message was that having a severe bleeding disorder did not mean that you could not achieve your goals in body art. It just may need planning with your HTC and adjusting treatment schedules.

As Scott said, 'Think Before You Ink'.

Australian haemophilia nurses were assisted to attend WFH World Congress through funding from a variety of sources. For more information, see page 12.

Image: Kevin Bidwell for Pexels.com

Jaime Chase is Haematology Clinical Nurse Specialist, Children's Cancer & Haematology Service, John Hunter Children's Hospital, Newcastle, NSW.

Exploring haemophilia prophylaxis

Yuhsuan Lin (Yoshi)

One of the most memorable sessions at WFH 2024 World Congress focused on the optimal dosage of prophylaxis treatment for individuals with haemophilia, highlighting the importance of this treatment in preventing bleeding episodes and joint damage. Discussions included varying dosages and frequency of prophylaxis, emphasizing the need for tailored approaches based on individual needs.

How much prophylaxis is enough?

Chairs ~ *Manuela Carvalho, Portugal; Emna Guider, Tunisia*

Adequacy of prophylaxis - What should be the target? ~ *Jan Blatny, Czech Republic*

The role of prophylaxis in personalized medicine ~ *Manuel Carcao, Canada*

Low-dose emicizumab ~ *Veena Selvaratnam, Malaysia*

In the session, the speakers mentioned that prophylaxis treatment dosages for individuals with haemophilia vary across different countries, with more economically developed countries often offering higher doses and more frequent treatments due to better access to resources and healthcare infrastructure. In contrast, lower-income countries may struggle to provide adequate dosages, resulting in less effective management of haemophilia and increased risk of complications. This disparity underscores the importance of addressing healthcare inequities to ensure all individuals have access to optimal prophylaxis treatments regardless of their economic situation.

Dr Jan Blatny, a consultant haematologist from Czech Republic, summarised the history of the goal and focus of haemophilia treatment to the present day.

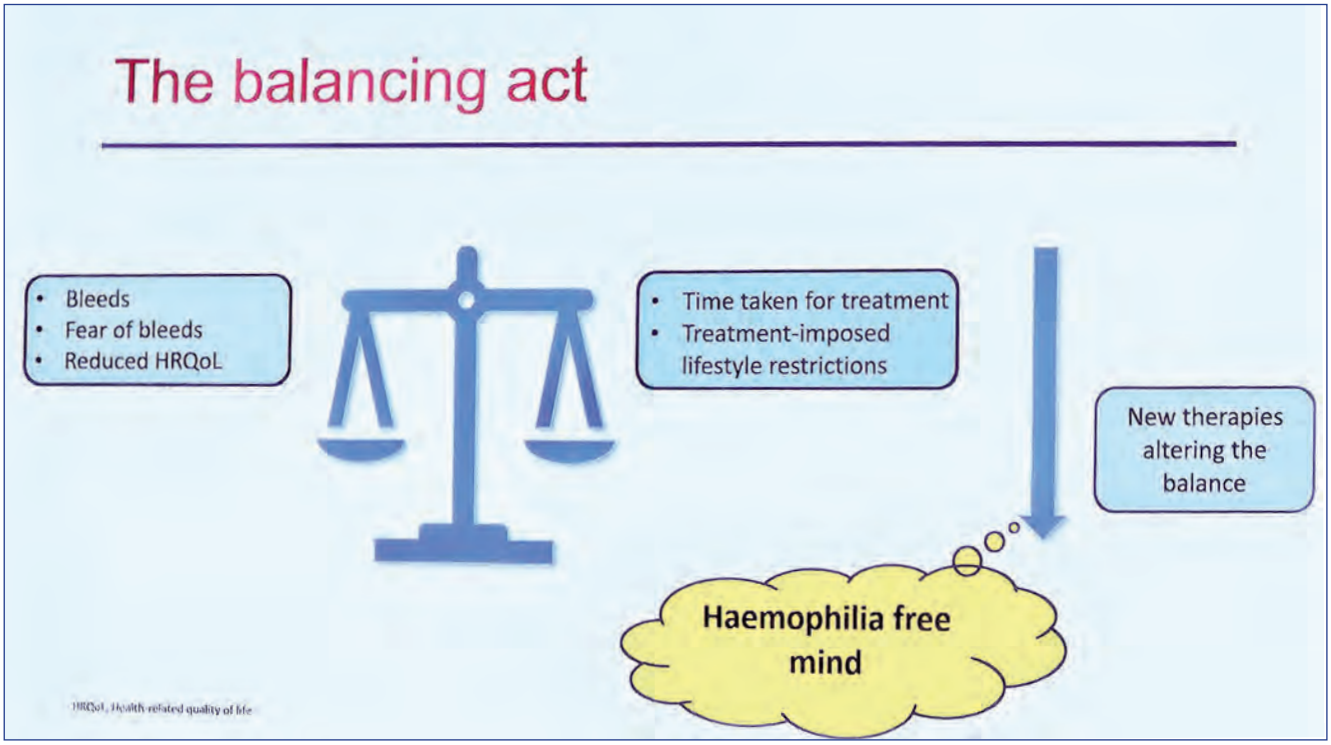
The initial haemophilia treatment goal was to respond to acute bleeds, often with episodic treatment. Then prophylaxis with a target of a factor level greater than 1% was introduced to reduce bleeding for patients with a severe phenotype, and later an increased target of a factor level above 3-5% was introduced to prevent bleeds and preserve joint health. He saw the evolution of treatments as moving from reducing bleeds towards eliminating bleeds.

I see this evolution of haemophilia treatment as being corelated to prevention as the core of health care. This is a crucial component of promoting overall well-being and reducing the burden of disease. It involves strategies aimed at preventing the occurrence or progression of illnesses through actions such as screenings, healthy lifestyle behaviours, and early interventions. By focusing on prevention, healthcare systems can help individuals maintain good health, reduce healthcare costs, and improve the quality of life for populations. Emphasizing prevention can lead to long-term benefits for both individuals and society.

In the WFH 2023 Comprehensive Care Summit, Dr Maria Elisa Mancuso, a haematologist from Italy, suggested - based on her research findings - that prophylaxis treatment should maintain a high factor VIII (8) trough level at 40% or above to achieve zero bleeds and that prophylaxis treatment should also be available for mild haemophilia patients. However, more intravenous infusions would be required to achieve high dose prophylaxis, which would also mean higher cost.

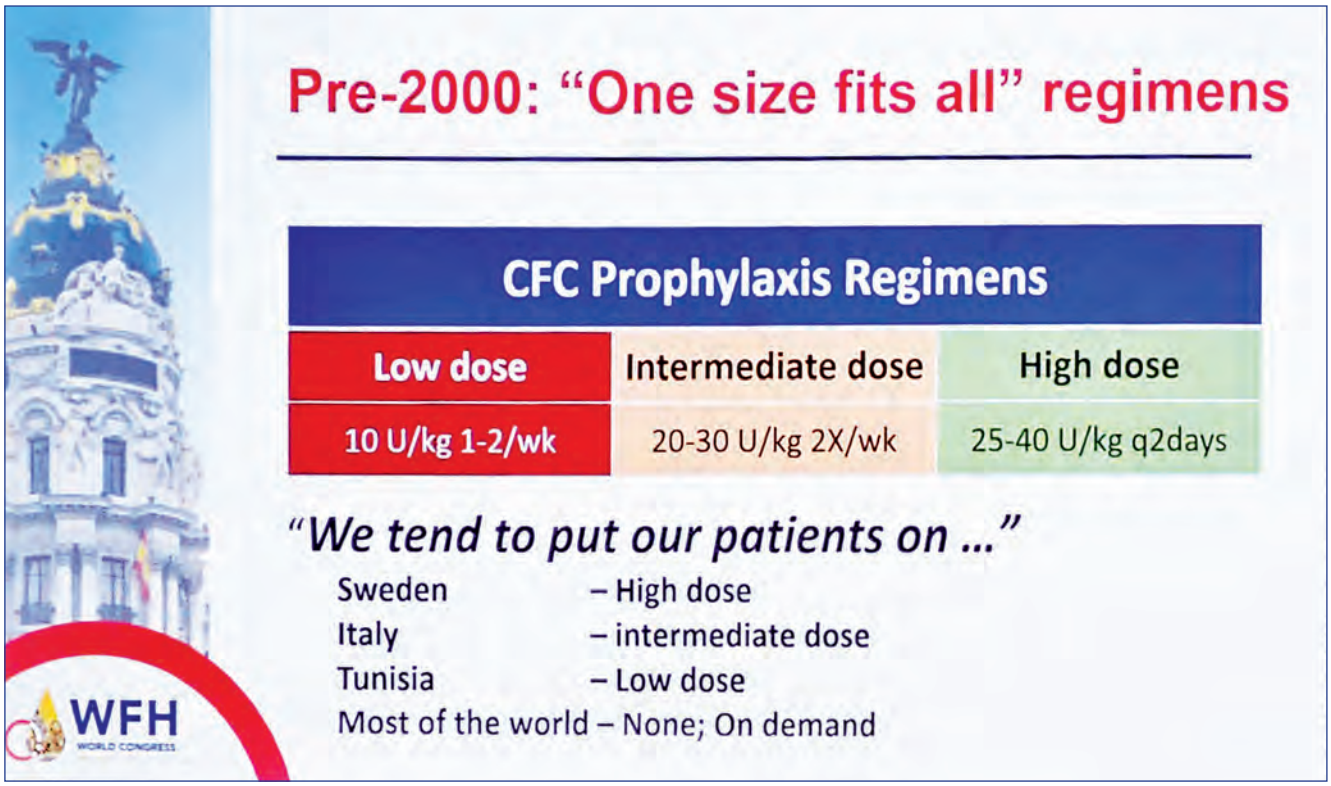
Dr Blatny questioned how much prophylaxis is enough, and are we able to achieve zero bleeds?

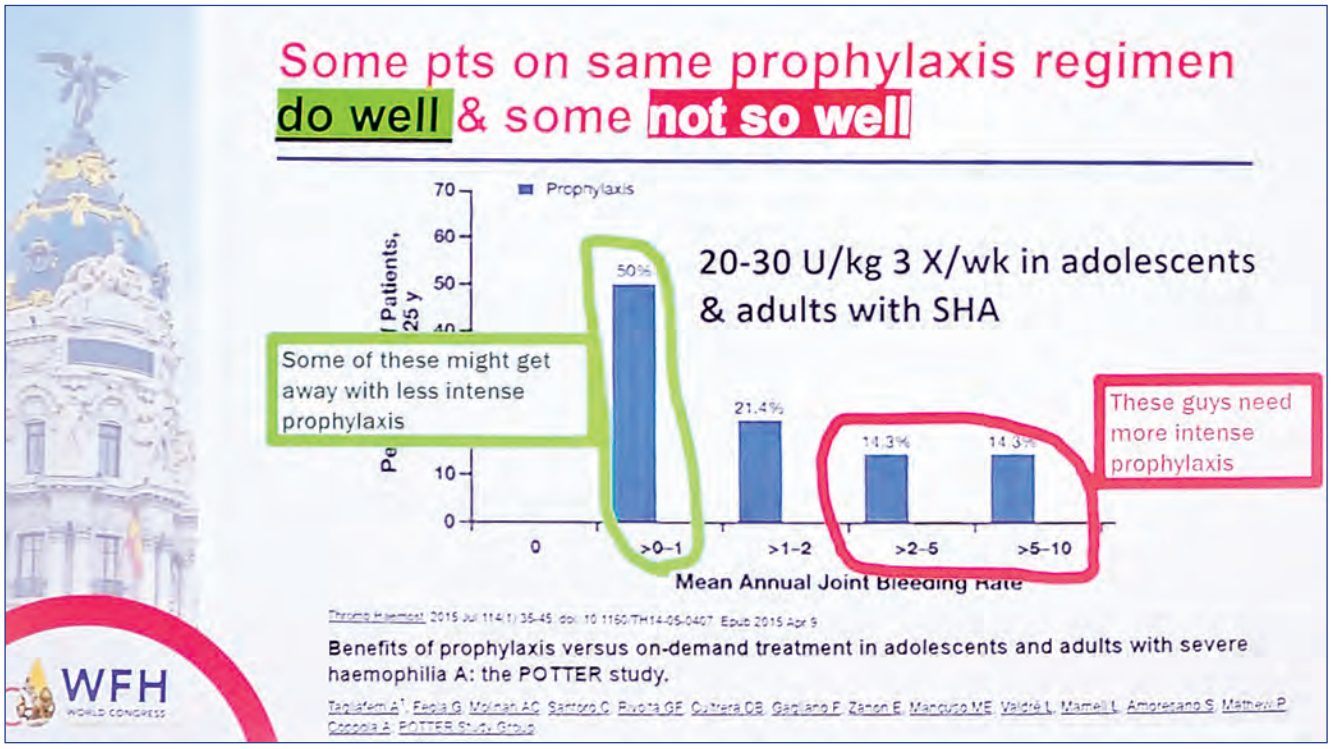
He presented his concept of the balancing act, showing that health care providers need to maintain a balance between the burden of bleeds and the burden of treatment on quality of life. He proposed a concept shift in treatment to hopefully achieve zero bleeds and better quality of life by offering treatment sustaining a high factor level, looking to further advances in non-factor therapy, rebalancing agents and gene therapy. >



Dr Manuel Carcao, a paediatric haematologist and Clinician Investigator from Canada, supported the idea of commencing prophylaxis treatment as early as possible to preserve joint health and to minimise bleeds. However, how should we achieve that with minimal treatment burden in order to have a better quality of life?

Before the year 2000, the practice was to put patients on 'one size fits all' regimens, with prophylaxis doses varying across different countries, depending on their economic status.





Dr Carcao pointed out this regimen did not always work as well due to the individual patients' factor levels, genetic and mutation types, plus the status of their joint health and levels of activity. He presented a study by Feola G, et al. 2015, *Benefits of prophylaxis versus on-demand treatment in adolescents and adults with severe haemophilia A: the POTTER study* which showed that patients had different bleeding rates and responses even when on same prophylaxis regimen. As a result, personalised prophylaxis treatment is now seen as the key to achieve patient centred care. That is reflected in our clinic, where treatment options and regimens are discussed in the yearly review with the specialist due to lifestyle variations, social economic status changes and bleeding episodes since the last clinic review.

Dr Carcao suggested that prophylaxis treatment should be started as early as possible to achieve joint health preservation and minimise bleeds.

However, he noted that prophylaxis treatment in haemophilia is expensive and around 80% of the world has very little or zero access to the products. Consequently, although low dose prophylaxis tailored to individual needs and affordability are not enough, they are much better than no prophylaxis. This approach still allows patients with haemophilia to reduce bleeding and slow the deterioration of joint health, leading to better lives.

Non-factor treatment is an option for personalised prophylaxis treatment, but raises questions such as the risk of thrombosis, cost and how individuals will handle the variety of medications when experiencing a bleed. Is low dose non-factor treatment an option for low to middle income countries?

Dr Veena Selvaratnam, a haematologist from Malaysia, presented the results on her low dose emicizumab studies, showing that low dose emicizumab resulted in zero bleeds in 9 out of 10 patients over 37- 293 weeks, and their QoL (quality of life) score improved significantly. Some other countries also showed positive outcomes with low dose emicizumab. Finland set a low maintenance dose and had zero bleeds in 10 out of 11 patients during 8-80 weeks. India also had zero bleeds in 8 patients who participated in low maintenance dose emicizumab, with an average trough factor level of 12.9% after 7 days. >

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Emicizumab is typically initiated with a loading dose schedule, which involves a higher dose for the first four weeks followed by a lower maintenance dose. Dr Selvaratnam stated there is no need to reload after this initial loading period unless specifically advised by a healthcare provider based on individualized factors such as response to treatment or treatment interruptions.

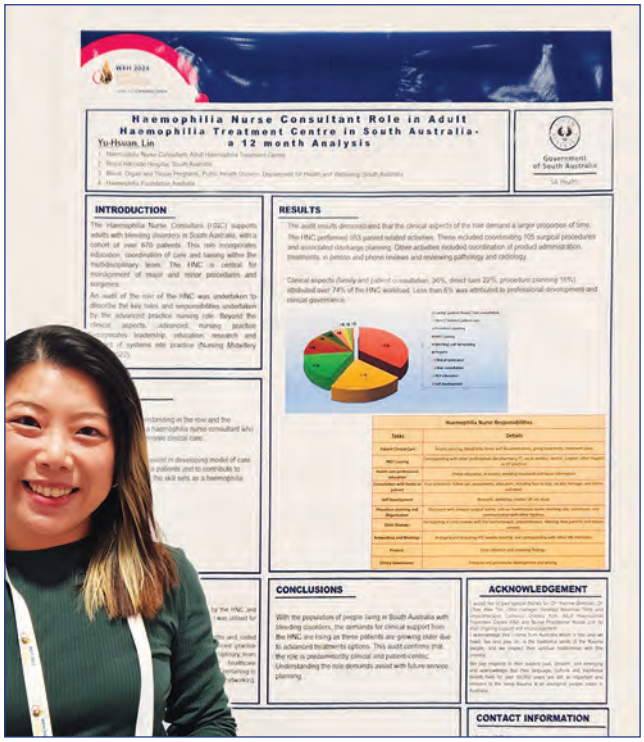
READ MORE

This session was published in an open access State of the Art review article in *Haemophilia* journal:

Carcao M, Selvaratnam V, Blatny J.
How much prophylaxis is enough in haemophilia?
Haemophilia. 2024;30(Suppl. 3):86–94.
https://doi.org/10.1111/hae.14964

HAEMOPHILIA NURSE ROLE POSTER

One of my posters was about the Haemophilia Nurse Consultant role in South Australia. This highlighted that Haemophilia Nurses play a pivotal role in ensuring comprehensive patient care and management for individuals with haemophilia. Our responsibilities include coordinating treatment plans, educating patients and their families about the condition and its management, monitoring, and managing treatment-related complications, facilitating access to resources and support services, and serving as a liaison between patients, healthcare providers, and other stakeholders. Haemophilia nurse consultants' specialized knowledge and expertise are essential in optimizing patient outcomes and quality of life while providing emotional support and advocacy for individuals with haemophilia.



I appreciated the opportunity to attend Congress with my South Australia Haemophilia Treatment Network team members - Senior Physiotherapist Cameron Cramey, ADBR Data Manager Venessa Mavrinac-Tiddy, and paediatric haemophilia nurse Daisy Regan.

I would also like to thank Haemophilia Foundation Australia for sponsoring my attendance at this wonderful event.

Yuhsuan Lin (Yoshi) was assisted by funding from HFA to attend the WFH 2024 World Congress.

Left to right: *Cam Cramey, Daisy Regan, Yoshi, Venessa Mavrinac-Tiddy*

Photos: Yuhsuan Lin (Yoshi)

Yuhsuan Lin (Yoshi) is Haemophilia Nurse Consultant at the Royal Adelaide Hospital, South Australia.

Women: caregivers to patients to advocates

Lara Nicholson

This session discussed the work of women advocates to overcome the general and longstanding misunderstanding that women are only carriers and do not have bleeding disorders, a notion that was challenged by the personal anecdotes and clinical case presentations at WFH 2024 World Congress. It explored how the idea that bleeding disorders are only experienced by men denies the impact on women and on their health services. The complexity of women's needs and their clinical presentations in this area of medicine have historically been largely dismissed or deprioritised. Hence the absolute cruciality of a collaborative partnership between Haemophilia Treatment Centres (HTCs) and women in the community and the Foundations that represent them.

How to go from caregiver to patient, and from patient to advocate?

Chair ~ *Pam Wilton, Canada*

The journey to self-advocacy – community testimonial ~ *Noemy Esther Diaz-Burgos, USA*

Overcoming psychological barriers ~ *Meila Roy, UK*

Creating supportive networks and advocacy initiatives ~ *Suzanne O'Callaghan, Australia*

SELF-ADVOCACY

Noemy Diaz-Burgos delivered a community testimonial as a woman with a bleeding disorder in the United States, relaying her personal experience growing up with symptoms and indicators she refused to let be dismissed. She spoke about her own personal experience becoming a career pathway where she is now actively pursuing change for women and girls with bleeding disorders in the United States and internationally, but noted that actually receiving a diagnosis was a long and challenging battle that required advocacy in itself.

Noemy Diaz-Burgos

Noemy described advocacy as varying from the act of speaking up, to representing the marginalised, to campaigning for change, to educating stakeholders to engaging your community and raising awareness.

Noemy was raised by a single mother from Puerto Rico and has a brother with severe haemophilia A. She initially advocated for her brother but eventually began advocating for herself to receive a diagnosis and a treatment plan that she said, even as a woman in her twenties, is still evolving as she works with her treating team to manage bleeding symptoms.

She spoke about her own challenges: worsening symptoms as she entered puberty, not being insured in the United States, experiencing a gender bias, gaps in education and being gaslit and >

misdiagnosed by health professionals – something that Noemy said fuelled her passion to campaign at a higher level.

She gave examples of the comments she heard from health professionals: *'but girls don't have haemophilia'*, *'it's just a heavy period'*, *'must be all the stress you're getting yourself into'*, *'you're too young to be worrying about this'*, *'stop trying to take the attention away from your brother'* and *'your symptoms can't be that bad'*.

Subsequently Noemy chose to follow up with speakers and attendees from bleeding disorders events, scheduled appointments with community-vetted doctors, documented and questioned everything and used handouts/toolkits for bleeding disorders organisations in her communication with health professionals and haematologists.

Noemy was validated when she was diagnosed with mild haemophilia A in November 2022 and could reconsider her treatment options.

Noemy then decided to intern with the US government and Hemophilia Federation of America (HFA) and researched pharmacy benefit management reform, inclusive language and genetic testing and began sharing her story as a powerful tool of advocacy. Noemy's advocacy efforts became her own career journey, and she has protested for reproductive rights in Washington DC, been on the Panel for the Department of Hispanic Health Services Secretary and Staff, trained advocates within the Florida bleeding disorders community and worked on Prison Health Reform.

Noemy emphasised her earlier point that advocacy can look different in different individual's contexts. Just being heard by a haematologist and receiving a diagnosis can be the most powerful outcome for a woman or girl with a bleeding disorder. Sharing the story of that effort and journey is an extension of advocacy work she encourages in other women and girls with bleeding disorders worldwide in their respective cultural contexts.

PSYCHOLOGICAL BARRIERS

Meila Roy is a Clinical Psychologist in the UK who works within the National Health Service (NHS) and acknowledged that her presentation draws on models and thinking developed in the West.

Left to right: *Suzanne O'Callaghan, Meila Roy, Pam Wilton*

Meila explored what we mean by a psychological barrier for an individual in the context of their person, their family, their healthcare and their society and referred to the Haemnet research group Cinderella studies by Kate Khair and colleagues in 2022, which aimed to understand the lived experience of women and girls with bleeding disorders in the UK. The study's main findings spoke to the difficulties experienced by women with bleeding disorders, even in a well-resourced country with a health care system that includes specialised bleeding disorder clinics. Difficulties obtaining a diagnosis, lack of awareness among health care professionals, and normalisation of symptoms by non-specialists were common.

Meila spoke of the pervasive notion that haemophilia is a condition that only affects males and the stigma around the discussion of women's health issues as being two of the barriers women and girls with bleeding disorders face in the UK, despite the fact that bleeding disorders affect both men and women – and that women experience additional, unique issues due to their biology.

Other prominent psychological barriers for women include guilt and blame of females and a lack of space for women's needs in this medical area. The study showed significant issues with access to care and consistent themes of feeling dismissed and minimized by health care providers as well as diagnostic delay, poor communication around

obstetric issues, misdiagnosis and gross health uncertainty. These outcomes all point to a lack of understanding of the health risks for women and girls with bleeding disorders.

Broader societal and cultural factors impacting on women and girls include caring responsibility/ burden, feelings of isolation and stigmatization.

Meila suggested a larger story about women and girls is being told within our community, a story that is '*changing, but not changed*'. She noted advocacy does not always look the same and her practice aims to empower women and girls to identify sources of support in their own context and equip clinicians to encourage this for our patients.

Finally, Meila pointed to several animated patient stories produced by the Cinderella team and available on the Haemnet YouTube channel (https://www.youtube.com/@Haemnet) including:

Struggles & Support: A mother's haemophilia story; The Cinderella Study

It's not all about boys: A story of haemophilia

Bruises, bleeds and babies: A story of Type 3 VWD

PEER SUPPORT AND ADVOCACY INITIATIVES

Suzanne O'Callaghan, Policy Research and Education Manager at Haemophilia Foundation Australia (HFA), spoke about the Australian experience of women's peer support and advocacy initiatives. She drew on the strategies and experiences of Australian women leaders as well as HFA initiatives for the content of this session and thanked the women who contributed to her presentation, who are patient advocates and peer support leaders.

Suzanne explored the trajectory for a woman from the first step of recognising she has a bleeding disorder, to self-advocacy and supporting her peers, and then to education and advocacy at a national or international level.

She began by describing the importance of stepping back and acknowledging women's peer support and advocacy strategies that have worked, honouring their wisdom, no matter how simple or obvious the strategy may appear to be. Sensitivity to local and cultural issues is pivotal in this area of advocacy as is observing and sharing what works in womens' individual environments.

What barriers can literally get in the way of diagnosis for women and girls with bleeding disorders? Apart from the issues discussed by the other presenters, Suzanne also mentioned that their symptoms can be put down to gynaecological problems therefore making them harder to define as a bleeding disorder. Relatively speaking, a woman's family might consider an amount of bleeding normal in their family, given this is their only experience of menstruation and this can lead to misconception of 'normal' bleeding.

Suzanne spoke about the awkward and culturally specific nature of discussions around menstruation, often making women and girls silent and also the additional comparisons women and girls may make to their male siblings regarding their severity of bleeding.

What we know about women socially and culturally are the priorities placed on family, partner, work and aspects of life other than their individual health and wellbeing. Suzanne noted this as a significant systemic barrier to diagnosis of bleeding disorders for women and girls. Finally, the simple difficulty in advocating for oneself when unwell and lacking energy is also a barrier for those living with chronic conditions.

Suzanne spoke of creating truly safe spaces where women and girls can have conversations that develop organically in an informal and relaxed environment, for example, with fun workshop-based activities, activities at community camps and women's brunches, as well as private online platforms. This supports child focussed conversations with women that can often evolve to discussion around women's bleeding problems.

One woman leader's experience was that *'women are more comfortable talking about their periods and bleeding with other women; I will openly talk about it to start the conversation; I'll talk about my experience with childbirth or with my period before I was diagnosed compared to now that I have treatment and that gets rid of the awkwardness'*. This conversation gave a clear message to women that *'you may be in a caring role but your health matters too'*.

Opportunities to raise the question about symptoms are important in identification and treatment and need to be done in a respectful and sensitive manner. HTC information sessions discussing mild haemophilia could be a good starting point, >

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as are regular collaborations between an HTC and a local haemophilia foundation to meet patients in clinic and develop peer support relationships with warm handover from HTC clinicians.

Cultural change is often challenging and Suzanne suggested drawing on the experience of change management in organisations. This approach speaks of '*bringing everyone with you', 'engaging hearts as well as minds*'. Change can be promoted by *'showing how'* with peer support, creation of safe spaces and managing the typical stages that are reactions to change - denial, resistance, anger and grief. And of course the importance of persistence and celebrating small gains. Suzanne emphasised the complexity and art of persuasion in this and how individual cultures will need to approach this differently.

In an evaluation of HFA's 'Female Factors' education resources, 50% of community members had given the resources to health professionals, family and friends and colleagues to educate them. Female Factors resources aim to augment the female voice. Suzanne noted that resources are evidence-based and high quality and include personal stories, tips and self-advocacy strategies shared by women in the community.

There are a number of tools that have worked for women and girls in Australia, including HFA Female Factors resources and the Canadian *'Let's Talk Period'* self-assessment tool. The Australian Bleeding Disorders Registry (ABDR) patient card is also standardised nationally to communicate with other health professionals - but we still need to advocate and ensure that women are registered in the ABDR.

Similarly, the evidence about women's experience is crucial and researchers are currently investigating worldwide to understand how bleeding disorders affect females.

Contributing to the evidence through the ABDR, the PROBE Australia study and other research is vital to inform data going forward.

Suzanne encouraged women to seek support in advocating for themselves and know who their allies are, such as family, partner, friends, health professionals from relevant areas and also connecting with male peers who have become 'champions' in the community.

Then looking systemically, Suzanne spoke of changing the conversation at every level; for example, the HFA Council's commitment that 'women and girls are a priority issue'. Social media, newsletters, information sessions and awareness campaigns need to repeat the message to the broader community and of course national conferences are an excellent forum for health professionals and community to share information and stories that aim to inform research and ultimately include women and girls alongside their male relatives in what is already a small and rare population.

Collaborations with wider health professional and community networks are crucial for this community, including schools, midwives and general practitioners (GPs) and this can be done by the simple sharing of a personal story in their professional education – to spark discussion and thought about women and girls with bleeding disorders and an evolving area of research.

Women leaders also need safe spaces and structures to connect, network, share skills and create their advocacy community. Bleeding disorders can impact on energy and ability to contribute and many women are also juggling other stressful life issues. We as a community need to consider this risk of burnout and support them to engage with their advocacy journey sustainably. This may be through the support and network of professionals and other women who can empathise with the complexity of their experiences.

Lara Nicholson was assisted by funding from HFA to attend WFH World Congress.

Photo: HFA

Lara Nicholson is Senior Social Worker at the Haemophilia Treatment Centre, Royal Brisbane & Women's Hospital, Queensland.

Women and girls with bleeding disorders

Suzanne O'Callaghan

There were several sessions on women and girls at the WFH 2024 World Congress, all with the strong message that it's time for action on female-specific research, treatment and care.

Women and girls with bleeding disorders

Co-chairs: *Paula James, Canada; Immaculada Soto-Ortega, Spain*

New haemophilia carrier classification - how do we adapt our care pathways?
~ Jill M. Johnsen, USA

Impact of therapeutic innovations on women and girls with hemophilia
~ Cedric Hermans, Belgium

HMB - from menarche to menopause
~ Nicola Curry, UK

One session that stood out for me was a medical session on women and girls with bleeding disorders where the speakers were discussing the nuts and bolts of how to make meaningful changes to the management of women and girls with bleeding disorders.

Dr Jill Johnsen, a physician scientist from the US, began the session by raising the concern at how few females predicted to need care have touched a Haemophilia Treatment Centre (HTC) – only 18% in the US, which is well-resourced and could expect to have a higher number of patients.

She proposed that there should be:

- Early diagnosis and care over the lifespan
- Testing for genotype at birth, confirming factor level as an infant
- Checking for bleeding risk and heavy menstrual bleeding.

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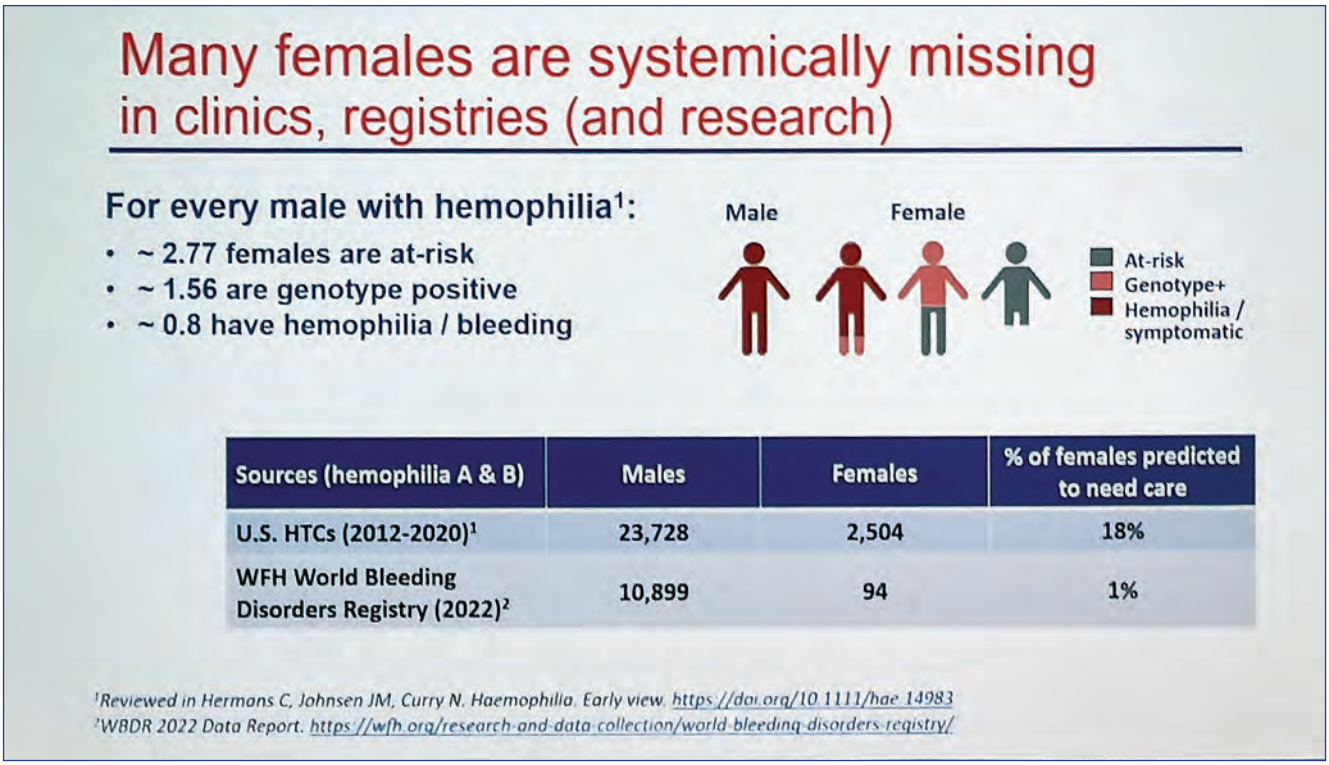

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Speaking about haemophilia treatment, Dr Cedric Hermans, President of EAHAD (European Association of Haemophilia and Allied Disorders), commented that the *'therapeutic revolution has so far been primarily male-centric, benefiting mainly boys and males with haemophilia.'* He pointed out that mostly females have mild or moderate haemophilia but there is very little treatment experience data. He proposed that:

- The treatment goals for females should be *'conversion into a mild or normal phenotype'* .
- Researchers need to identify agents that can lead to normal haemostasis in females - nanobodies could be very useful.
- Females need different endpoints to males in clinical trials – and there needs to be clinical trials specific to females.

Dr Nicola Curry, a haematologist from Oxford in the UK, followed him with a discussion of heavy menstrual bleeding (HMB) and the severity of its impact on women and girls with bleeding disorders. Iron deficiency, for example, is a major problem and has a negative effect on quality of life.

In the UK HTCs have developed a Menarche Plan, where they discuss what is normal and what might occur at menarche (first menstrual period). However, the challenge for women with bleeding disorders is that periods may become heavier over their lifetime. She underlined that there needs to be an option for patient-initiated follow-up and women invited to contact the HTC when they have problems.

The speakers and audience debated how best to record and assess menstrual bleeding. Nicola Curry noted that tools to document menstrual bleeding such as Pictorial Blood Assessment Charts (PBACs) are important, but they need to reflect new forms of sanitary protection and be available electronically, eg as an app. A member of the audience asked about having one standard Bleeding Assessment Tool (BAT) for diagnosis internationally. Cedric Hermans responded that there still needs to be work on this but a good first step would be to use existing BATs. Australia's Dr Janis Chamberlain also pointed out the quality of life evaluation tools that have been developed by her team in Newcastle.

The session finished with a discussion about research and treatments for females with bleeding disorders – that is a need for:

- More research into effective treatment for females with VWD.
- A different approach to physiological bleeding such as heavy menstrual bleeding
- Challenging the exclusion of females from clinical trials due to hormonal treatment for heavy menstrual bleeding.
- *'We need to do better.'*

READ MORE

This session was published as a State-of-the-Art review article in *Haemophilia* journal –

Hermans C, Johnsen JM, Curry N. **Women and girls with inherited bleeding disorders: Focus on haemophilia carriers and heavy menstrual bleeding**. Haemophilia. 2024;30(Suppl. 3):45–51. https://doi.org/10.1111/hae.14983

Suzanne O'Callaghan was assisted by funding from WFH and HFA to attend WFH World Congress.

Suzanne O'Callaghan is HFA Policy Research and Education Manager

New and emerging issues in physiotherapy

Abi Polus

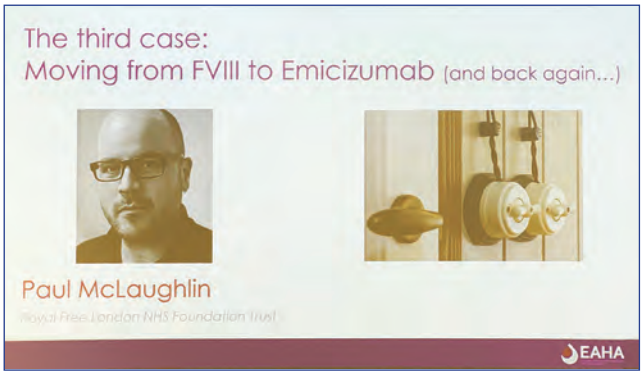
I was extremely lucky to be supported to attend the **17th Annual Congress of the European Association for Haemophilia and Allied disorders (EAHAD)** meeting in Frankfurt in February 2024. I felt it was important to attend further education as the management of haemophilia and other bleeding disorders is rapidly changing and it is vital that clinicians keep up with the changes in real time so that we can best treat the people we look after.

CHANGES TO BLEEDS

I am seeing people with bleeding disorders in the haemophilia clinic and on the hospital ward who are having a different presentation of bleeding to what we had traditionally seen. People who are on non-factor therapies, extended half-life products, and gene therapy now report significantly fewer bleeding episodes, but bleeds they do have can be more difficult to identify, or as a few of my patients have described as 'feel weird'. This can lead to late presentation and an increased period of dysfunction. We are also seeing increased presentations of people who are having muscle bleeds and we need to consider why this may be.

It was extremely interesting to attend the Congress and note that this is an issue around the world and that many, many musculoskeletal health professionals and other members of the multi-disciplinary team are all seeing this. Lectures and formal and informal discussions all discussed this phenomenon.

An entire day prior to the Congress was devoted to physiotherapy management and was extremely informative for me. An interdisciplinary session combined with nursing and psychosocial services had a discussion of non-factor therapies, with very relatable and pertinent case studies, leading to an interactive and informative discussion about what we are now seeing and how it has changed. In particular, and extremely relevant was senior physiotherapist and patient advocate Dr Paul McLaughlin from the UK demonstrating a case of joint decision making in choosing treatment and presentation of musculoskeletal issues. >



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Further talks by some key Haemophilia Physiotherapy researchers addressed perspectives on how bleeding has changed with new therapies (Prof David Stephensen, UK), difficulties in recognising bleed or not bleed (Merel Timmer, Netherlands) and a further case presentation interview with a patient using non-factor therapy (Paul McLaughlin, UK).

LATEST RESEARCH

This was followed by a SLAM session, where 10 minute presentations of 6 recent musculoskeletal related trials were presented. These were very interesting and included using light-up pods to improve balance and reaction time, developing an allied health program with an occupational therapist (which I have personally been advocating for, for a long time!!). Utilisation of local physiotherapists (those outside haemophilia treatment centres/ HTCs) in the Netherlands was discussed. I agree with this concept, but it made me smile that they emphasised that some patients had to travel up to an hour to get to their centre, where we know that is minimal for most of our patients, where 4 hours or more to access HTCs is not unusual. A systematic review of outcome measures was interesting.

The SLAM session I found of most interest was one that was using evidence to co-design (physio and patients) to develop a virtual-based program that aims to increase confidence to exercise for people with haemophilia by Stephanie Taylor, a UK-based physiotherapist. She described the collaboration with patients about what they really wanted in an exercise program, which was then delivered. The final session was a return to sport lecture by Dr Thomas Hilberg (Germany), using principles of healing times and stressing the need to find the delicate balance of not returning too early so re-bleeding and re-injury risk is high, and not too late where loss of range and strength and muscle and bone mass may be difficult.

Throughout the rest of the congress there were various interesting sessions with themes including von Willebrand disease (VWD), women and bleeding disorders, novel therapies, acquired haemophilia, artificial intelligence (AI) and pain. The following is a summary of those sessions of particular interest.

ARTIFICIAL INTELLIGENCE (AI)

An AI Session by various doctors was very informative, with the message that this is not a topic that we can ignore or shy away from: it is the present and the future and is going to be around and we need new skills and literacy in AI. We are using technology with electronic medical records, registries, telehealth, apps, internet platforms and various other media. We need to use it with ethical principles of transparency and responsibility. Prof Mike Makris, a haematologist in the UK, was enlightening in his talk on AI in scientific communication and gave us quite frighteningly real examples of deep fakes - and the trust we may need to challenge. One example he gave is there could potentially be a circulating video of him/us with same face/voice - saying something that he/we have never spoken about. They could have us saying anything - how would we know what is truth? He stated that we cannot avoid it by ignoring it; it is already mainstream.

He also discussed Generative pre-trained Transformer (Chat GPT) and how it can be used ethically and how it should not be used. He highlighted the inadequacies in AI: in general it is prone to mistakes and cannot currently assess quality. It can 'hallucinate and fabricate' (i.e., make things up) and AI tools do not cite their sources and make up references. He told us of a site called Retraction Watch that has tracked 14,000+ papers retracted in last 5 years. This is great in that papers are being authenticated, but worrying that we may read something incorrect that has been made up and not yet validated. All peer-reviewed professional journals have rules on AI and you have to declare use of AI in a research paper. We need to use AI ethically and vigilantly.

JOINT HEALTH AND PAIN

An excellent session was on joint health in haemophilia in an evolving treatment landscape.

Dr Annette Von Drygalski (USA) discussed the clinical implications and joint bleeding, joint remodelling, chronic pain and reduced quality of life (QOL) and noted that poor joint health is a globally reality. Dr Hortensia de la Cortez-Rodriguez (Spain) discussed current joint health measures and emerging challenges including the subclinical bleeding being undetected. In a study of people with non-severe haemophilia A and no history of a joint bleed, haemosiderin (residual blood) deposits were found in 14% of people who did not report any symptoms. >

Prof David Stephensen (UK) discussed the biomechanical and neuromuscular changes in joint and muscle bleeds, the perception of pain and psychological impacts. He reiterated that pain does not directly correlate with structural damage. He presented various studies in haemophilia that addressed this. He discussed the study by McLaughlin who interviewed patients with pain regarding their views of management and exercise and of note mentioned a patient comment of '[it is] difficult to score out of 10 as something you live with', which certainly made me think and consider my practice and questioning.

IMPACT OF NEW THERAPIES

A session on non-factor therapies for haemophilia and VWD by Dr Steve Pipe (USA) discussed new therapies like emicizumab and some in advanced clinical trials. Dr Andreas Tiede (Germany) discussed change in acquired (non-hereditary) haemophilia, which was very interesting. A session on the optimised outcome of haemophilia treatment, 'Towards a haemophilia free mind' by Dr Cedric Hermans (Belgium), included thought on people with haemophilia who may no longer bleed needing physical coaching to get them where they want to be. In discussing long term joint health, Dr Roberta Gualtierotti (Italy), noted that originally >1 % of circulating factor level was thought to be protective, then 3-5%, and now it is considered the factor level may be even be higher. She discussed synovitis (inflammation of the joint lining) and the unmet needs we still have - tools to collect early detection of joint damage.

Professor and physiotherapist David Stephensen highlighted the need for more uniform assessment tools and identified the need for more meaningful and guided rehabilitation. He is involved in current research - which I have been contributing to – and is aiming for a core set of performance-based tests to monitor physical function across lifespan. He has identified 7 tests - tests that are widely used in other musculoskeletal conditions. Another outstanding talk was by Anna Wells, a UK-based physiotherapist, who explored post-traumatic stress symptoms and pain memories in people with haemophilia and the influence on their current pain. It was extremely interesting. She found perceptual and emotional parts of pain were difficult to verbalise. People with haemophilia could recall and relive pain and trauma even in presence of no painful stimuli. She suggested that HTCs need to be trauma informed and management may need to include reprogramming memories. She discussed how psychosocial input can be extremely valuable here. This may become even more relevant in the future for children who may not have ever needed intravenous (IV) access but may in the presence of a rare bleed when they are in an acute situation that is already scary and painful.

I was also able to discuss ideas for research and general patterns of presentation with my global colleagues and took comfort in we are all experiencing similar issues with novel therapies and bleed identification, different bleed presentation to what we have experienced previously and different management, and various other changes. I was able to bounce ideas for collaboration and be inspired by the amazing research my colleagues are doing overseas and ponder how we may do similar in Australia to identify need and improve services in Australia.

I am extremely grateful to have been supported in having the in this opportunity to attend, which has reassured me that my work with patients is similar to my colleagues around the world and can challenge me to improve my skills and better help those around me.

Abi Polus was assisted by funding from Sanofi to attend EAHAD 2024.

Photos: Abi Polus

Stock photos: Ryutaro Tsukata and This Is Engineering for Pexels.com

Abi Polus is Senior Clinical Physiotherapist – Haemophilia at the Ronald Sawers Haemophilia Centre, Alfred Health, Melbourne

New developments in haemophilia nursing

Megan Walsh

I was very fortunate to be selected by the Australian Haemophilia Nurses group to be supported to attend the **17th Annual Congress of the European Association for Haemophilia and Allied disorders (EAHAD)** in February 2024. It has been over 7 years since I have attended an overseas conference and it was so exciting to see what new developments have been happening in the haemophilia sphere.

The Congress took place over 4 days with over 2000 delegates. This is a snapshot of some of the sessions I attended.

IMPACT OF NEW THERAPIES

The first day commenced with a multidisciplinary session with physiotherapists, nurses and psychologists discussing the experiences with the new subcutaneous treatment product emicizumab (Hemlibra®) for haemophilia A. It was interesting to hear we were all seeing similar experiences in patients using this product – for example, that a lack of adherence was causing significant muscle bleeds and children were complaining that the subcutaneous injection stings. Several interesting case studies were presented by a nurse, a physiotherapist and a psychologist and discussed by the group.

LATEST RESEARCH

There was also a SLAM session where five nurses who have submitted posters were selected to speak on their research. Topics covered included training staff on reconstitution of clotting factors, looking at an adult transition program, an analysis of an electronic health record used for recording health data by patients in clinical trials, and analysis of nursing practices in HTCs in France.

There was a focus on the need for more psychology input for the bleeding disorder patients undergoing new treatment trials. Speakers emphasised shared decision-making, especially when discussing enrolment in trials or commencing new products. There was an interesting panel discussion around treatment choices and what multidisciplinary support was required to help in patient decision-making.

Assessing health literacy of patients and tips for dealing with patients with general low literacy were also discussed. The session explored what aids were required and that visual aids to explain complex information were often useful and what they would look like.

WOMEN

The needs of women with bleeding disorders, their diagnosis and management were also highlighted. It was noted that there are at least two potential carrier females related to each male with haemophilia and there are also women with other types of bleeding disorders, but Haemophilia Treatment Centres are not seeing the numbers of women that would be expected. There was discussion around how to encourage these women to seek diagnosis and treatment at Haemophilia Treatment Centres.

ARTIFICIAL INTELLIGENCE

The session on Artificial Intelligence (AI) was interesting and many examples of the uses of AI in different fields were presented. AI can be a powerful tool. It is starting to be used in diagnosis and individualizing patient treatment in some diseases. It potentially can analyse a lot of data quickly and identify trends or patterns so it may be useful for clinical trials and research. It was interesting to hear all articles submitted to medical journals are now scanned for AI input. Speakers also showed the other side of AI, where fake news and non-existent people could be created. AI brings many ethical challenges around privacy, data protection, societal impact and even its use in medical care.

NEW TREATMENT PRODUCTS

There were presentations on clinical trial results for many new treatment product studies, including one for recombinant VWD, a once-a-week extended half-life recombinant factor VIII, and second generation gene therapy trials. Among other novel therapies discussed were another product called mim8 which mimics factor VIII much like emicizumab, but more potent, along with fitusiran, concizumab and SerpinPC, which are all new subcutaneous drugs that prevent bleeding, targeting different parts of the clotting pathway and rebalancing it. Several of these trials involve Australian patients.

MILD HAEMOPHILIA

Mild haemophilia was another area discussed. It was noted patients with mild haemophilia tend to present late for treatment or not at all and thus were living with complications of bleeds. We do see inhibitor development in this group of patients as there are specific genotypes that are associated with a higher risk of inhibitor development. So in this session there was an emphasis on the need for patients to have their factor levels checked and genetic testing to identify inhibitor risk, and also to present earlier for treatment of bleeds.

EAHAD 2024 was a very stimulating four days and a great experience, and certainly very beneficial for my practice.

Australian haemophilia nurses were assisted to attend WFH World Congress through funding from a variety of sources. For more information, *see page 12.*

Abi Polus and Megan Walsh at EAHAD

Photo: Abi Polus

Megan Walsh is Clinical Nurse Consultant at the Ronald Sawers Haemophilia Centre, Alfred Health, Melbourne

AHCDO research: VWD and acquired haemophilia A

Caitlin Rice

Dr Caitlin Rice is a fifth-year trainee from Northern Ireland. Caitlin has been working in Australia on an Out of Program Fellowship with the aim of developing clinical experience outside the UK NHS system and gaining further research experience in non-malignant hematology and bleeding disorders. In 2023, Caitlin was located at Royal Perth Hospital with Prof Wendy Erber with a focus on laboratory hematology. In 2024, she transferred to Fiona Stanley Hospital with Dr Stephanie P'ng, commenced in the role of AHCDO Research Fellow and started planning research projects.

Two of Caitlin's research projects are outlined below. Caitlin will work closely with the AHCDO ABDR Research Fellow to utilize data from the ABDR for these projects.

Type 3 von Willebrand disease in Australia: review of outcomes

Type 3 von Willebrand disease (VWD) is a rare bleeding disorder caused by a marked deficiency or absence of von Willebrand factor (VWF). It accounts for < 5% of all patients with VWD and occurs with an approximate incidence of 1 in every 500 000 individuals. According to the most recent report of the Australian Bleeding Disorders Registry (ABDR), there are 148 patients registered with the ABDR who are classified as having severe (type 3) von Willebrand disease.

The management of type 3 VWD is complex and variable. Management guidelines are usually embedded within guidelines of other forms of VWD including the much more common type 1 VWD and there are no guidelines specifically addressing this complex disease.

Primary Outcome

To characterise the clinical and laboratory features of patients in Australia with type 3 VWD and document the current clinical care focussing on the use of prophylaxis and the burden of bleeding events.

Secondary Outcomes

To identify unmet needs in patients with type 3 VWD including treatment options and long-term disease burden.

The Australian experience of haemostatic management in acquired haemophilia A

Acquired haemophilia A (AHA) is a rare and very serious acquired bleeding disorder. The aetiology is of autoantibodies against factor VIII (FVIII/8), which impair FVIII function.

Standard of care treatment utilizes immunosuppressive therapies to suppress autoantibody formation. Another mainstay of treatment is the prevention of injury with risk of subsequent bleeding, and use of bypassing agents to control active bleeding. Haemostatic therapies include recombinant activated factor VII (rFVIIa), activated prothrombin complex concentrate (APCC) or recombinant porcine FVIII (rpFVIII). As haemostatic therapy is expensive and complex, its use should be guided by experienced specialists.

These agents are used irrespective of inhibitor titre and residual FVIII activity - meaning that many patients with life or limb-threatening bleeding will require use of bypassing agents, and often over an extended period of days to weeks. A recent article in *Blood* journal (2021) addressed the use of emicizumab as an alternative haemostatic agent in the management of AHA. The use of emicizumab may have significant cost saving implications due to good haemostatic efficacy, allowing early discharge, and avoidance of expensive bypassing agents for haemostatic control.

Primary Outcome

Record the extent of product use for haemostatic management of AHA in Australia over 5 years (2018-2023).

Secondary outcomes

Quantify the clinical and economic burden of bleeding complications related to choice of haemostatic agents in acquired haemophilia A management.

This may inform cost effectiveness for a business case for consideration of use of emicizumab as first-line therapy for haemostatic management in patients in Australia with AHA.

Photo: Roger Brown for Pexels.com

Dr Caitlin Rice is the 2024 AHCDO Research Fellow

FACTORED IN
FOR YOUNG PEOPLE

Catching up with youth

Local Foundation youth catch-ups are a great way to connect with other young people. Declan and Bailey talked to Haemophilia Foundation Victoria (HFV) about the HFV Youth Day Out event at the gaming venue, Fortress Melbourne, and what made it fun.

What did you enjoy most about the Youth Day event at Fortress Melbourne?

The interaction with others of a similar background and the ability to feel included and welcomed.

Declan

I enjoyed hanging out with the group and being in an environment where it's just the youth group all together.

Bailey

How did gaming contribute to making the event fun and exciting?

It made us all interact with each other and gave us all something to bond over.

Bailey

Declan and Bailey's story

Did you meet any new friends or make new connections during the Youth Day event?

I'd like to say that everyone I met on the day were lovely individuals and new friends.

Declan

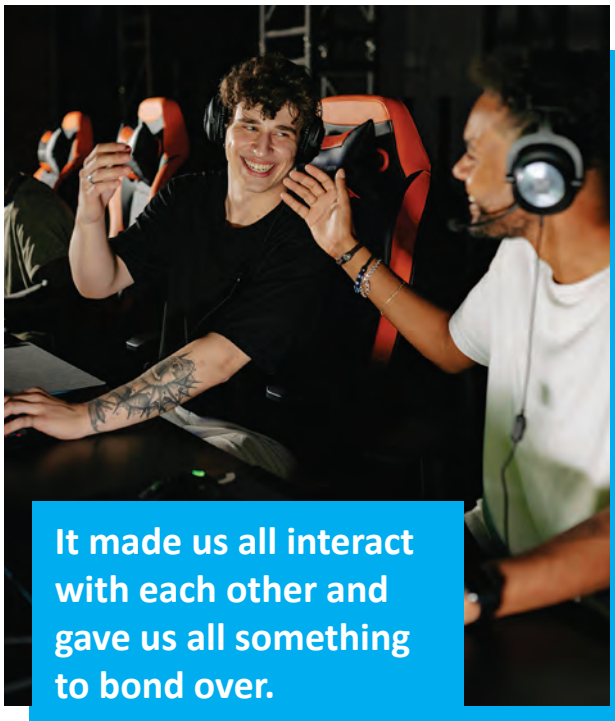
Did you feel supported by your peers during the event?

Yes, because we all acted in a group and there was no negativity.

Bailey

Definitely, everyone was eager to interact and socialise, creating a welcoming environment.

Declan



It made us all interact with each other and gave us all something to bond over.

How would you describe the atmosphere at Fortress Melbourne during the event?

Despite myself not having a gaming background, I felt included and happy due to the character of each individual and felt the atmosphere suited the particular event.

Declan

Would you attend another event like this in the future?

Definitely, positive past experience can only mean more to come.

Declan

What message would you like to share with other youth about attending events like this?

It's great fun and a must-do thing, everyone gets along great, u get to have amazing chats and have fun as a team.

Bailey

This article is adapted and reprinted with permission from 'Voices of the event: HFV's Youth Day Out through the eyes of attendees', The Missing Factor, Winter 2024, magazine of Haemophilia Foundation Victoria (www.hfv.org.au).

Stock images: Yan Krukov and A Darmel for Pexels.com

Find out more

For more information about youth events, contact your local Haemophilia Foundation. You can find contact details on the HFA website at https://tinyurl.com/Aust-Foundations

CALENDAR

World Hepatitis Day
28 July 2024
www.haemophilia.org.au/world-hep-day

Bleeding Disorders Awareness Month
October 2024
www.haemophilia.org.au/BDAM

World Haemophilia Day
17 April 2025
wfh.org/world-hemophilia-day

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