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National Haemophilia

www.haemophilia.org.au

WFH 2022 World Congress

Montreal and virtual

8-11 May 2022

WFH 2022 World Congress

Hear the latest from the International experts

Bleeding Disorders Awareness Month

How to be involved

World Hepatitis Day

Stories of hep C and being cured

HAEMOPHILIA FOUNDATION AUSTRALIA

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HFA is now on TikTok!

To celebrate, we're running a video challenge and we want you and your family to join in the fun.

It's simple - grab a fact from our 'About Bleeding Disorders' poster, record a video about it, and share it online. And in the process, help raise awareness for bleeding disorders!

Australian submissions received by **30 September 2022** could win one of a few **$50 vouchers**.

FIND OUT MORE

Visit https://tinyurl.com/HFA-video-challenge

Gavin Finkelstein

President, Haemophilia Foundation Australia

From the President

Here we are already in September, and it's time to be preparing our annual reporting either at our state/territory Foundations or for the HFA annual general meeting which is coming up in late October.

I have recently written to the Hon Mark Butler, the new Minister for Health and Aged Care, to welcome him to the portfolio. We are currently seeking to renegotiate grants so that we can continue to provide governments with advice and national peak body services to our community. I have also recently contributed my experiences to a government consultation about improving public transport and parking for people with joint and mobility problems.

LIVING WITH COVID-19

We have all been going through challenging times with COVID-19 still impacting, even if in different ways from the first two years of the pandemic. It is exciting that some of our Foundations are planning face-to-face events for this end of the year and we hope they will go ahead without interruption. We know how much our peer support groups and camp attendees like to get together and for some it has now been a few years since they have seen each other. Many of our health professionals have not been able to take breaks and we think of them as they have continued to provide services for our community.

We have learned new ways of communicating and to do things differently and I doubt we will return to our old normal. You will see in this issue of *National Haemophilia* that the 2022 WFH World Congress was a hybrid face-to-face and virtual event – and indeed, some of the sessions discussed the ongoing impact of COVID-19 and virtual health.

I think it's fair to say that many people in our community have learned to communicate with their health professionals at their Haemophilia Treatment Centre via telehealth, when they had not anticipated this could be successful. We need to be able to connect effectively to our Haemophilia Treatment Centre specialists, and of course also having a local General Practitioner who is also in the loop is critical to our best care plan.

NEW HFA DIGITAL COMMUNICATIONS

If you use the HFA website regularly, you will see we are making changes all the time, and we hope the new information is helpful. Some people may not have seen some of the sessions from our 2021 national Conference. We are pleased that we've been able to make some of the presentations or parts of the presentations available on the website so you can catch up. You will also see a variety of other new education resources and information that might be of interest to you and family members. I recommend a browse on the website www.haemophilia.org.au.

Younger people in our community might also be interested in the information and personal stories on our youth website www.factoredin.org.au. You may have noticed that we are venturing into new territory on our social media platforms, with short videos, Reels – and now TikTok! We are celebrating our new TikTok platform with a competition to flush out the talent in our community – check the details on our HFA and Factored In websites.

We are working hard to make sure all parts of our community have the information, resources and representation they need.

Bleeding Disorders Awareness Month

OCTOBER 2022

As you may know, this year in October we will have a whole month to raise awareness about bleeding disorders.

The 2022 theme for Bleeding Disorders Awareness Month is **ONE COMMUNITY, MANY FACES**. Each week we have different topics and will meet community members from all ages and stages of life to hear their stories.

ACTIVITIES AND EVENTS – BOTH VIRTUAL AND FACE 2 FACE

We will launch our calendar at the end of September. To be advised about events, register for our E-news www.haemophilia.org.au/enews or keep an eye on our website and social media platforms.

PROMOTIONAL ITEMS

Promotional items orders are now open. Order the free goods to use for your school, workplace or family/friend event. We have balloons, pens, Fling Things (like a frisbee), colouring-in sheets, information posters and lots more.

Put your order in at
www.haemophilia.org.au/BDAMorder

HOW CAN YOU BE PART OF THE WEEK?

- Order promotional items for your event, information stalls and your child's school/child care
- Run a virtual or face-to-face fundraising event
- Host a red cake day
- Share information on your social networks
- Share your story - https://tinyurl.com/HFA-story
- Children and their friends can take part in the colouring-in competition or a Scavenger Hunt
- Take part in HFA and Foundation activities (Calendar coming soon)

For more information, contact
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WFH 2022 World Congress

The **WFH 2022 World Congress** was held by the World Federation of Hemophilia in Montreal, Canada on 8-9 May 2022 as a hybrid event, both in person and virtually.

It was an opportunity for the international bleeding disorders community - including people with bleeding disorders, health professionals, national member organisations and industry - to come together to discuss current issues, share knowledge with colleagues, and problem-solve with delegates from diverse backgrounds.

So much has changed recently: radically different haemophilia treatments and the COVID-19 epidemic have impacted enormously on the community. There continue to be great strides ahead in managing women and girls with bleeding disorders and there is new research into VWD and the very rare bleeding disorders.

In this issue of *National Haemophilia*, we hear from some of the Australians who attended. Shauna Adams participated in person as a Youth Delegate on behalf of HFA. Haemophilia nurses Jaime Chase and Robyn Shoemark, physiotherapist Aisha Barton-Ross and Suzanne O'Callaghan from HFA attended virtually.

Thanks to all for their contributions.

WHAT DID THE DELEGATES SAY?

'For the first time, Congress sessions were offered face-to-face and virtually. Hopefully this has allowed people who may normally not be able to travel internationally to attend Congress. Having sessions available live and on demand means you can view at your own leisure. I watched sessions on demand as concentrating between 1 and 4am in the morning was just too much for me to handle and then still come to work for the day.'

Robyn Shoemark, Clinical Nurse Consultant Haemophilia/Haematology, The Children's Hospital at Westmead

WFH national haemophilia organisation training

Shauna Adams attended the World Federation of Hemophilia (WFH) global national member organization (GNMO) training at the WFH World Congress in May 2022. She participated as a Youth Delegate, representing HFA. She spoke to Suzanne O'Callaghan at HFA about her experience.

Suzanne: Where was GNMO Training held?

Shauna: GNMO Training was held in conjunction with the WFH World Congress this year, at the Palais de Congress in Montreal. Some sessions were held at the hotel nearby where the GNMO participants were staying, so we were all in close quarters. We were located within walking distance of old Montreal, with lots of unique architecture and access to the local cuisine.

Chateau Ramezay Museum Montreal

Suzanne: What was different about GNMO Training this year?

Shauna: Feedback from previous congresses indicated that there was a level of disconnect from the GMNO training to the congress itself, so this year WFH trialled a new delivery method where the GMNO training occurred in conjunction with the Congress. Participants were provided with a suggested schedule of Congress sessions to attend that complemented their training. This kept the group together throughout the week and really allowed us to solidify our new connections.

There was a big focus on reconnecting as a group in person for the first time since the pandemic began. Some of our peers were unable to make it through the current visa processing system, so as a result we received a mixed method delivery of both virtual and online sessions. With a few technical bumps along the way, for the most part the technology enhanced the experience for those attending in person, along with access to simultaneous interpreting services. With a group from such a variety of different countries, the interpreting service meant that those who did not speak the language of the presenter could remain engaged with the presentations in real time. It also allowed people to ask questions in their native language to be translated back to the presenter.

I attended the Congress as a Youth Delegate, and the opportunity to meet and network with other young people in the community was invaluable. I was also able to meet some delegates in person for the first time whom I had connected with online some time ago – a common experience across the board with the travel restrictions we have been facing recently.

Suzanne: Did any sessions stand out for you?

Shauna: We had the opportunity to meet over lunch with all the Asia Pacific NMOs, chaired by our regional manager Guada. I heard about everyone's current priorities, and we took the time to touch base after a few years of no face-to-face contact. As a Youth Delegate this was a new experience for me, though there were several other new faces in the

group too. I look forward to meeting with this group again and explore collaboration opportunities in the future.

I really enjoyed the 'Ask the Experts' session as part of the GMNO training, where four delegates from Malaysia, Panama, India and South Africa spoke about unique challenges in each of their locations. The strong leadership shown by each of the speakers and the way they have all overcome adversity was really inspiring.

Suzanne: What was the most memorable aspect of the World Congress?

Shauna: Attending the first Women's Networking Event chaired by Dawn Rotellini. We celebrated the achievements of women who have been making a significant contribution to the bleeding disorders community, including our own Susie Cooper from HFWA. The Susan Skinner Memorial Award recipients were introduced and presented with their awards by Susan's son. It was really interesting to hear about how the landscape has changed since Susan started advocating for safe treatment for her sons.

It was lovely to see such a huge focus on women with bleeding disorders throughout the whole Congress, with an entire stream of sessions addressing topics around genetic testing, taboo topics, and increasing awareness for treatment of women with bleeding disorders. There was a real shift from talking about women simply as carriers, as we have in the past, to opening the scope to carriers, women with haemophilia and VWD, mothers of children with bleeding disorders and women with other rare bleeding disorders. Many experiences were explored, and all were presented equally.

Suzanne: What did you like about the social aspects of Congress?

Shauna: It was great to meet with people socially face-to-face for the first time for quite a while. I met people who are doing amazing things for our community which has really inspired me to think about what more I can do after a period where we were really all just trying to get through the post pandemic 'normal'. There was a lot to see in Montreal. I love to sightsee via the food options so I sampled a lot of the local cuisine – including sampling many bagels! It was great to network with other Youth Delegates and talk about ideas for the function of WFH in the future.

Shauna at the WFH General Assembly

Suzanne: What were the take home messages?

Shauna: We had a big focus on the WFH mission: **Treatment for all**. It was a timely reminder as we move into the world of exciting new long-life products and gene therapy to remember those who may not yet have access to reliable sources of what some of us may consider bare minimum treatment options. The pandemic has really highlighted the disparity in our community where some have had to prioritise treatment for COVID-19, or transportation and supplies were redirected to other areas.

Suzanne: Why do you think the World Congress is important?

Shauna: Congress brings us all together and provides an opportunity to refocus on what is important, what our priorities are as we transition into an age with gene therapy and long-life products. For those of us who are fortunate to receive world class treatment, it is important to understand the challenges of the global community and consider how we can support the WFH mission of Treatment for all.

Shauna Adams was funded by HFA and WFH to attend the WFH World Congress.

Shauna Adams is HFACT Secretary and an Australian community member with VWD.

Suzanne O'Callaghan is HFA Policy Research and Education Manager. *Photos: Shauna Adams*

Moving on from COVID-19

Jaime Chase

Plenary - Moving on from COVID-19 as a global community

Speaker ~ Brian O'Mahony, Ireland

Brian O'Mahony, CEO of the Irish Haemophilia Society, provided a thoughtful plenary on how the bleeding disorders community can move on from the initial phase of the COVID-19 pandemic. He outlined the community priorities and unmet needs prior to COVID-19. His discussion then evolved to new trends in Haemophilia Treatment Centres (HTCs) during the pandemic and the impact experienced by staff, patients and volunteers. Finally, he explored moving forward from the pandemic and the effects that it will have on the community.

BEFORE COVID-19

Prior to the COVID-19 pandemic, the priority of the bleeding disorders community was access to safe, sufficient and affordable therapy for all people with haemophilia and other bleeding disorders. Brian O'Mahony also explored new and novel therapies and the effects of these on the population. However, he pointed out there were continued unmet needs in the population, especially regarding community members with rare bleeding disorders, people with haemophilia who are ageing and women with bleeding disorders.

NEW TRENDS IN HTCS

Clinical practice changed vastly during the pandemic. Telemedicine had been trialled in the past but had never been embedded into clinical practice as the preferred option. Telemedicine allowed clinical assessments to continue, avoided in-person attendance and enabled the patient to maintain contact remotely with their HTC. Some centres were offering remote phlebotomy and electronic prescribing, which further enabled the physical dependence on HTCs to decrease. Physiotherapy could be offered via telemedicine, which greatly increased patients' adherence and also enabled the introduction of online physical conditioning programs.

IMPACTS ON HTCS AND SUPPORT ORGANISATIONS

There was a great increase in requests for telemedicine and the ability to provide outreach services in this way. HTCs had to pivot to provide care within this environment and brainstorm ways to make this transition as useful and as easy for patients as they could. Meetings were held virtually and support organisations switched to working from home. Virtual meetings and conferences were held, which increased registrations and participation but it has been noted that the ability of participants to network with colleagues and peers decreased.

MOVING FORWARD WITH COVID-19

Brian O'Mahony discussed the promotion of telehealth as a viable, equivalent option for all at length, and highlighted its various positive points. HTCs now have the ability to offer a range of appointments and treatment options to suit individual circumstances when providing comprehensive care. Video triage as a viable option for injury management and the provision of offsite phlebotomy would also be advantageous for HTCs.

He recommended that organisations providing meetings and education opportunities should offer the events as a hybrid event to encourage both virtual and in person attendance.

Jaime Chase is Haematology Clinical Nurse Specialist at the John Hunter Children's Hospital, Newcastle, NSW

Virtual care

Aisha Barton-Ross

The COVID-19 pandemic has brought virtual care - both challenges and benefits - to the forefront of healthcare, which was a key theme in a number of sessions I attended at the 2022 WFH World Congress in Montreal. Poignantly, the Congress was held as a hybrid online and face-to-face event this year in light of ongoing travel restrictions brought on by the pandemic. Two sessions covered some highly relevant issues in expanding virtual haemophilia care.

Hot topic debates -
Tele rehabilitation: yes or no?

Chair ~ Adolfo Llinas, Colombia

Speakers:

Yes *~ Peter Aguero, Physiotherapist, University of California, San Diego, USA*

No *~ Mark Krimmel, Physical Therapist, Washington Center for Bleeding Disorders, Seattle, USA*

The pros and cons of tele-rehabilitation were debated in the Hot Topics session. The speakers emphasised that virtual care is not a 'one size fits all' model but demonstrates clear benefits for some patients. They discussed a number of benefits and limitations of tele-rehabilitation:

Benefits	Limitations
• Improves access to care • Less disruptive to the home environment • Favourable for patients more comfortable in their home environment • Time efficient • Cost efficient	• Issues with technology • Issues with language translation • Lack of 'hands on' feedback • Less available objective measures for use • Safety and privacy concerns

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Peter Aguero took the 'pro' position and highlighted how telerehabilitation fits into the realm of evidence-based practice.

Source: What is evidence-based practice? Simmons University Library Guide, 2012 - simmons.libguides.com/ebsw

I thought this was a great way of highlighting that providing the best evidence-based care does not simply rely on what best research is available. At present, the body of research evidence behind face-to-face assessment and treatment is greater. However, given that virtual care provision is still emerging, that does not discount the merit of virtual care, especially when the patient's needs, available resources and the environmental context are considered. The discussion following this session also highlighted that although virtual care should be considered another 'tool in the toolbox', it needs to be individualised to the to the patient and their situation.

Use of technology with patients

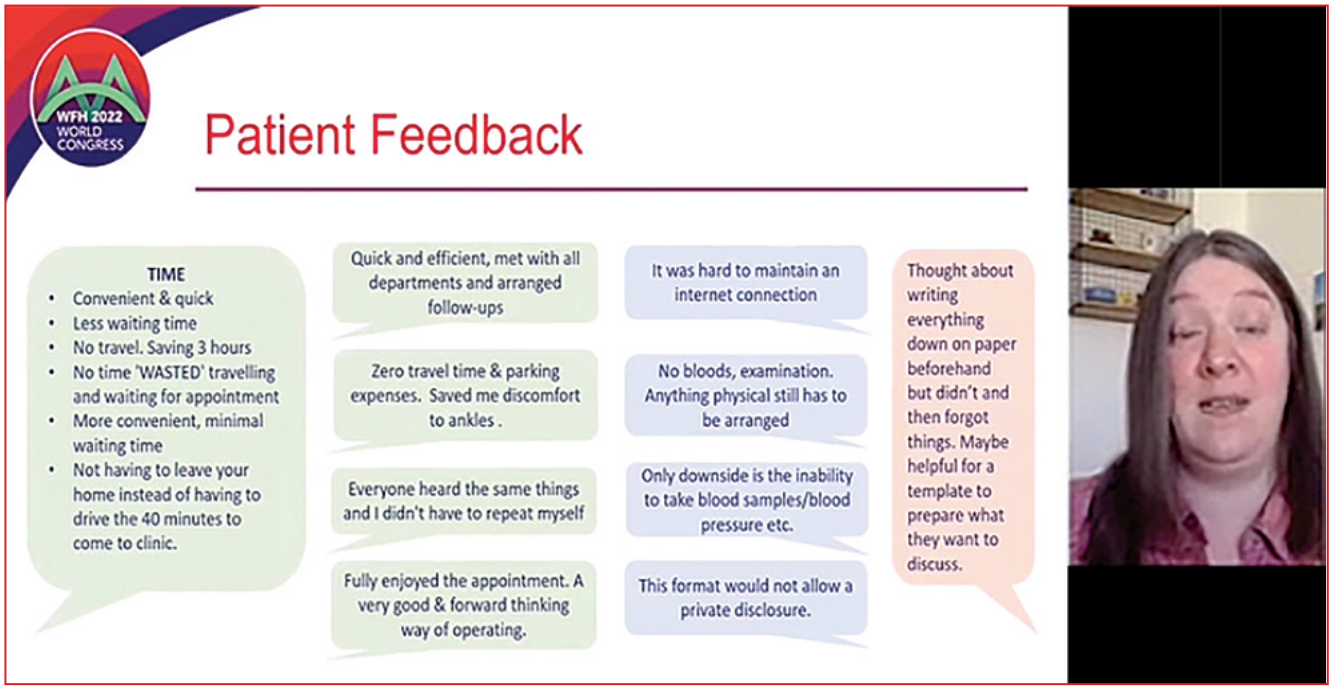
Chair ~ Marlène Beijlevelt, Hemophilia and Research Nurse, Amsterdam UMC, Netherlands

Telemedicine ~ Helen Manson, Haemophilia Sister, Belfast Health and Social Care Trust, UK

Helen Manson outlined how a virtual model of care had been integrated into the haemophilia space and talked about the steps involved in launching a novel virtual multidisciplinary team (MDT) haemophilia clinic from the Northern Ireland Haemophilia Centre in Belfast in 2020.

In January 2020, nursing and physiotherapy teams started work with the hospital IT department to explore a proof-of-concept study prior to launching a pilot virtual MDT clinic. This included developing resources for both clinicians (Microsoft Teams training, a video consultation etiquette guide, and a clinic script to standardise key assessment measures) and patients (information leaflet and telehealth guide) to ensure both parties were prepared for the virtual clinic format. Electronic patient and clinician feedback forms were also developed across the project. The pilot clinic was launched in May 2020, in somewhat opportune timing, shortly after COVID-19 restrictions were put in place. Once feedback from the pilot clinic had been analysed, the full virtual MDT clinic was launched.

Five virtual MDT clinics with a haematologist, nurse, physiotherapist, and social worker were then held between May and July 2020, with 28 patients participating. Interestingly, compared with an audit of face-to-face clinics across a comparable time period, attendance rates were more than 20% higher in the virtual clinic and the mean time per consultation was more than halved. Though feedback from patients was extremely positive, benefits and limitations mimicked those discussed in the previous sessions.



Feedback about the virtual clinic – *Slide published with permission from Helen Manson, Belfast Health and Social Care Trust*

I thought this model highlighted two key elements both physiotherapy and wider multidisciplinary health services should target when considering a virtual care model: ensuring adequate training and preparation for both clinicians and patients, and ensuring feedback is regularly encouraged so that the service can continue to be refined.

The take-away messages from these sessions for me were:

- We should embrace a hybrid of both face-to-face and virtual care provision, where both are individualised to the patient and their situation.
- Education, training and resources for both the patient and clinician should be used to improve the virtual care experience.
- Patient and clinician feedback should continue to be sought to identify barriers and challenges in virtual care to enable these to be addressed.
- Identifying those who would benefit from virtual care allows re-allocation of time and resources to those more vulnerable who would benefit from ongoing face-to-face care.

FURTHER READING

Sayers F, Manson H, Brennan B, et al. Virtual consultations: Providing alternative ways of supporting patients with inherited bleeding disorders. Haemophilia 2021 Jul;27(4):e498-e501. doi: 10.1111/hae.14210. Epub 2020 Nov 27.

Aisha Barton-Ross is a physiotherapist with the haemophilia team at the Royal Children's Hospital, Melbourne

Living in the new world of novel haemophilia therapies

Robyn Shoemark

Discussion about the impact of novel therapies for haemophilia was a highlight at the WFH Congress. This included non-factor therapies such as emicizumab (Hemlibra®) and fitusiran and gene therapies for haemophilia A and B.

At the time of publication, fitusiran and gene therapy for haemophilia were not registered by the TGA in Australia as prescription medicines.

CLINICIAN PERSPECTIVE

Surgery in an era of novel therapies

Chair ~ BJ Ramsay, Clinical Nurse Specialist, Blood and Cancer Centre, Wellington Regional Hospital, New Zealand

I was fortunate to present in the nursing session on surgery when a patient is being treated with novel therapies, along with Jaime Chase from John Hunter Children's Hospital in Newcastle. In this session nurses from around the world discussed case studies of patients using new treatments and Jaime and I represented Australia with our patient case studies.

It was great to hear about the patients on emicizumab having surgery and the treatment regimens they used. When there is little information or limited use of the new treatments, seeing what someone else has tried can help clinicians make an informed decision when you are planning treatment or surgery for your patient.

Changing treatment, changes diagnosis

Chair ~ Kate Khair, Director of Research, Haemnet, London, UK

What PWH tell us about living in the new paradigm of hemophilia treatment

~ Simon Fletcher, Lead Haemophilia Research Nurse, Oxford Haemophilia And Thrombosis Centre, UK

Non-factor therapy in persons with acquired hemophilia A and von Willebrand disease

~ Ming Lim, Assoc Prof, Division of Hematology and Hematologic Malignancies, University of Utah Health, Salt Lake City, USA

Patient panel:

Life with hemophilia after gene therapy

~ Luke Pembroke, Creative Director, Haemnet, London, UK

Life with hemophilia with fitusiran

~ Louis Marlow, biotechnology researcher, University of Edinburgh, UK

Life with hemophilia on emicizumab

~ Andrew Selvaggi, haemophilia advocate, Haemophilia Foundation Australia, Melbourne, Australia

In the first session, Simon Fletcher presented his view on how changes in treatment have changed the lives of his patients. He was careful to start by saying that he did not have haemophilia and that he is a treater. His role was to undertake and review studies aiming to find out what it means to have haemophilia and then use that information to help patients.

He described current treatments available for patients as aiming to **Replace**, **Rebalance and Replicate**. He grouped the studies into two categories: one he called **Emi and Me** for patients on emicizumab and the other **Exigency** for patients undergoing/who have undergone gene therapy.

In these studies, people with haemophilia made the following comments about what changes/ improvements in treatment meant to them:

Freedom – patients gained freedom and felt they and carers were liberated with new treatment options.

Control – patients and carers being able to plan for the future. In some cases, this was the first time they had ever felt able to plan for the future.

Reduction in pain – many patients were experiencing less bleeds so in turn, less pain.

Reduced burden – newer treatments being quicker and easier to administer allowed more time for life.

Side-effects – for example, in gene therapy known side-effects include needing to take steroids but this is still better than having to do prophylaxis.

Old Montreal *Photo: Shauna Adams*

ACQUIRED HAEMOPHILIA

Ming Lim then presented on non-factor therapy in people with acquired haemophilia A.

Acquired haemophilia is a rare autoimmune disease predominantly diagnosed in the elderly, where immunoglobulin (IgG) antibodies, also known as inhibitors, attack factor VIII (8). It presents often with severe bleeding and has a high morbidity and mortality often associated with gastrointestinal bleeds.

Past treatment was to control and prevent bleeding using bypassing agents or recombinant porcine factor VIII. The goal is to control bleeding and eradicate the inhibitor. Therapy includes the use of steroids and/or immunosuppressive therapies.

There have been several reports of off-label use of emicizumab. Ming Lim reported on a review of 24 patients using emicizumab to help control bleeding. Dosing varied from the same as that recommended for people with haemophilia A to a modified version of dosing. The duration of treatment ranged from 20 days to 10 months with 100% of patients reporting no further bleeds after commencing emicizumab. Clinical trials are ongoing.

PATIENT PERSPECTIVE

In the patient panel, three men told us their story of their journey with changing treatment for their haemophilia.

Luke from London spoke to us from the jungle in Peru where he has been for the last month. He underwent gene therapy in February 2020. He now has factor levels around 20-25% and has not needed any factor in the past two years. He has taken emergency factor with him to Peru and the only time he touches it is when he changes out the ice packs to keep it cold.

The presentation made Luke reflect on the last two years and what it has meant for him. As he underwent the treatment one month prior to COVID lockdown in the UK, he not only had a change in his treatment but found himself moving to be close to the hospital so he could reduce his risk for getting COVID and continue his hospital visits as part of the trial he was on. This was difficult for both his physical and mental wellbeing. He felt unwell with the side-effects of the medications he needed to take post gene therapy. He thought his veins would get a rest but with all the blood tests, this was not an option for the first 12 months. Moving on, he now only has visits every 6 months and his veins are finally having a rest. >>

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He talked about how gene therapy has changed his life for the better. He is able to plan things for the future. He felt he was unable to do that prior to gene therapy as he never knew when he might bleed and need to be near the hospital. He no longer thinks about haemophilia every day. Going to Peru has been a road test for his therapy. He does still have ankle pain from previous joint bleeds, but he knows the difference between pain and bleeding and is able to rest, take his pain medication and keep going the next day.

In the Q & A, when asked if he would do it again, he replied absolutely yes. He felt the trial process outlined the risks versus benefits and he had a very good consenting process so he would strongly advocate for trials and collection of long-term data associated with trials. He is very positive and has no regrets but did say he would be very disappointed if the effects wore off and he had to return to having factor again.

Louis from Scotland talked about life with haemophilia on fitusiran. Louis was diagnosed with severe haemophilia B at 18 months and then went on to develop an inhibitor. He was then treated with the bypassing agent NovoSeven® (recombinant factor VIIa). When tolerisation was attempted, he had an anaphylactic reaction. He has undergone radiosynovectomies for his target joints.

In 2019 he joined a trial using fitusiran for his prophylaxis. Other than developing asthma, which is a known possible side-effect of the medication, Louis is very pleased with his new treatment. He has gone from having approximately 9 bleeds per year down to 1 bleed per year. He has been able to increase his activity levels and finds that his bleeds are now injury/trauma related rather than spontaneous. He has gone from regularly needing to use a wheelchair to now the wheelchair is taking up space in the garden shed. He is very happy to be on a new therapy.

Andrew from Australia talked about life with haemophilia on emicizumab. Andrew was diagnosed with severe haemophilia A at 15 months and by the age of 2 years had developed an inhibitor. By 2007 at age 20 years, he had had over 700 joint/muscle bleeds, had developed 7 target joints and was wheelchair bound from 5-18 years of age. He underwent a personal transformation and lost 30kg.

In 2016, he participated in the Haven clinical trial and had his first dose of emicizumab. The life changes for him have been immense. Since starting on the trial, he has not had any muscle or joint bleeds. Since commencing emicizumab he has undergone orthopaedic surgery again. In past surgeries, it had been difficult, 'full of bleeding' but this time it was controlled with less bleeding.

His described the challenges as both mental and psychosocial and that he needed to reset his expectations after starting the trial. He found he needed to build confidence and start trusting his body, something he had not been able to do pre-trial.

He did mention that while he strives to do his treatment as recommended, sometimes he forgets, likening it to putting out the bins. We all forget regular things sometimes. He is, after all, only human like the rest of us!

CURRENT RESEARCH

Late-breaking Clinical Research

Chair ~ Glenn Pierce, Vice President Medical, World Federation of Hemophilia, USA

A phase 1 sequential pharmacokinetic (PK) evaluation of octocog alfa, rurioctocog alfa pegol, and efanesoctocog alfa in severe hemophilia A

~ Annemieke Willemze, Senior Clinical Research Director, Sanofi Genzyme, Amsterdam, Netherlands

I attended the Late Breaking Clinical Research session where Annemieke Willemze discussed the BIVV001 (efanesoctocog alfa) trial results. These are very promising for people with haemophilia A, with a 3-4-fold half-life increase to an average of 43.3 hrs and requiring a weekly treatment injection.

Robyn Shoemark is Clinical Nurse Consultant Haemophilia/Haematology at the Kids Factor Zone, The Children's Hospital at Westmead, Sydney, NSW

Gene therapy

Gene therapy for haemophilia has been in advanced clinical trials for some years now and this Congress was a timely forum to discuss and evaluate it critically.

Robyn Shoemark

Plenary: Perspectives on curing hemophilia

Speaker ~ Mark Kay, Professor of Pediatrics and Genetics, Stanford University School of Medicine, USA

In his plenary presentation *Gene therapy for haemophilia - Where are we towards a cure?* Professor Mark Kay outlined the progress in gene therapy to date, what's in the pipeline and some key questions for the future.

Since the 1980s there has been a lot of progress and, more recently, clinical success in gene therapy.

Virus vectors engineered in the laboratory have evolved to become efficient carriers to deliver gene therapy in humans. The AAV (Adeno-associated virus) vector has been found to be effective in delivering the sequences needed in gene therapy for haemophilia.

In the first haemophilia B gene therapy trials there was a T-cell response causing transaminitis (high levels of certain liver enzymes called transaminases) and expression of factor IX waned. In the clinical trials that followed, steroids were used and helped to reduce the transaminitis.

Mark then went on to discuss gene therapy clinical trials for both haemophilia A and B. In future we can expect to see gene therapy involving genome editing technology, with directed changes to the host's DNA sequences, and other different types of gene therapy. Watch this space.

A significant hurdle to overcome in future trials is the differences between animal and humans. This includes dosage, expression and safety. Mark posed a number of questions:

- How do we predict human outcomes? There is variability in individual dosing and immune responses.
- Are we targeting the correct cell types in the liver? Hepatocytes vs liver endothelium.
- What is the risk for cancer? Oncogenesis, or the process where normal cells turn into cancerous cells, is likely a low but not an absolute zero risk.

Where are we going from here? Mark concluded there is room for improvement in AAV vectors, along with new vector possibilities. Genome editing with gene transfer technologies is in the pipeline. Gene therapy may result in lifelong expression from neonate to adult, with the possibility of reinfusion to 'fine-tune' dosing.

Late-breaking Clinical Research

Chair ~ Glenn Pierce, Vice President Medical, World Federation of Hemophilia, USA

Immune suppression following gene therapy in hemophilia

~ Wolfgang Miesbach, Leiter Schwerpunkt Hämostaseologie/Hämophiliezentrum, Universitätsklinikum Frankfurt, Germany

Wolfgang Meisbach discussed some of the immune complications of gene therapy for haemophilia A and B and their implications. He highlighted that patients need to be aware of both the risks and benefits before undertaking these therapies. Known complications include transaminitis and cellular immune responses. This is seen in the form of raised AST/ALT (liver function) and IgG (Immunoglobulin G antibody) results which can be treated with corticosteroids.

There are still many differences in clinical trials and their outcomes, including variability in factor levels. How appropriate is gene therapy for an individual patient? It is important to weigh up the risks vs benefits to decide what is right for the patient and if a trial is indicated for them. >>

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Gene therapy continued

Suzanne O'Callaghan

Plenary: Gene therapy - are we ready now?

Speaker ~ Radek Kaczmarek, Postdoctoral Research Associate, Gene and Cell Therapy Group, Wells Center for Pediatric Research, Indiana University School of Medicine, Indianapolis, USA

In a thoughtful but challenging presentation, Radek Kaczmarek continued the discussion about the current gene therapies for haemophilia A and B that use AAV vectors to transfer the gene. He explored the concerns about efficacy and safety and the pathways to lessen the side-effects and find solutions to these issues, including for those who are as yet unable to receive gene therapy.

He commented on some current concerns:

- High liver responses in some patients
- More durable response in haemophilia B gene therapy than haemophilia A.

Most patients who received gene therapy have shown an increased factor level and have remained off replacement factor therapy. However, the variability in factor levels is a concern as those with suboptimal levels cannot have a repeat dose for 15 years.

He also proposed that there needs to be much more discussion about the ideal target factor level range for gene therapy. For example, factor levels over 15% virtually eliminate joint bleeds.

In conclusion, he argued: 'Overall the causes and mitigants of wide variability, waning efficacy in the case of haemophilia A and liver toxicity have not been sufficiently studied.

'Due diligence around uncertainties will be critical for people with haemophilia, who have endured a difficult safety legacy and failed hopes. Ideal gene therapy for haemophilia will ultimately close the gap between how much haemostatic correction the therapy can provide and how much is needed to provide a life independent of treatment. The current state of the art brings us close to this goal, but getting there will involve more time and innovation.'

Please note that the Congress reports above represent the views of the Congress speakers.

At the time of publication gene therapy for haemophilia is available through clinical trials. Some advanced clinical trials are promising and some gene therapy treatments may well reach the global market in the next year or so if they pass the required regulatory hurdles.

If you want to know more about clinical trial opportunities for you, it is recommended that you speak to your Haemophilia Treatment Centre doctor.

Suzanne O'Callaghan is HFA Policy Research and Education Manager.

Robyn Shoemark is Clinical Nurse Consultant Haemophilia/Haematology at the Kids Factor Zone, The Children's Hospital at Westmead, Sydney, NSW.

Women and girls with haemophilia

Robyn Shoemark

Changing treatment, changes diagnosis

Chair ~ Kate Khair, Director of Research, Haemnet, London, UK

Recognizing women with a bleeding disorder: a new diagnosis

~ Sarah O'Brien, Assoc Prof Pediatrics, Director of Experimental Therapeutics, The Ohio State University, Nationwide Children's Hospital, Columbus, USA

Sarah O'Brien's presentation at Congress tackled some of the issues in diagnosing haemophilia in females.

She commented that haemophilia diagnosis for women has previously been ambiguous. Some practitioners have frequently been heard to say that females affected by haemophilia do not bleed and that their male relatives' bleeding disorder has nothing to do with the females' bleeding. We as bleeding disorder health care professionals know this is not true.

Girls and women affected by haemophilia need to be diagnosed correctly. If their factor level is greater than 40%, the diagnosis is *symptomatic* or *asymptomatic* carrier, depending on their bleeding phenotype (the detectable expression of the gene or their clinical symptoms). If their factor level is less than 40%, the categories of *mild*, *moderate* and *severe* that are given to males also apply to females.

In clinic, history taking is of utmost importance. There is wide variability in bleeding phenotypes amongst females. There is often no correlation between factor levels and bleeding. Females often present with heavy menses (periods), oral cavity bleeding, postpartum haemorrhage (severe vaginal bleeding after childbirth) and excessive bleeding after dental extraction. Joint bleeds and subclinical bleeds can occur. This is why clinical phenotypes are more important in females than actual factor levels. Genetic testing should be part of the haemophilia diagnosis process for females.

The take-away from this session was the questions to use when talking with a females in clinic when abnormal bleeding is suspected. Does the female bleed more than 7 days when she menstruates, have more than 4-5 changes of sanitary products daily (make sure to clarify the products used as there is a large range of absorbency in products on the market), need to change products at night, do they have gushing when they stand up, and are there any clots and are they larger than the size of a grape? Answering yes to these questions would warrant further investigation to lead to a correct diagnosis. And don't forget that obligate haemophilia carriers may have other bleeding disorders too.

Robyn Shoemark is Clinical Nurse Consultant Haemophilia/Haematology at the Kids Factor Zone, The Children's Hospital at Westmead, Sydney, NSW.

Von Willebrand disease

Suzanne O'Callaghan

Old Montreal *Photo: Shauna Adams*

Plenary (Arosenius Lecture) - Towards novel treatment options in von Willebrand disease

Speaker ~ Peter Lenting, Director of Research, French National Institute of Health and Medical Research - INSERM, Paris, France

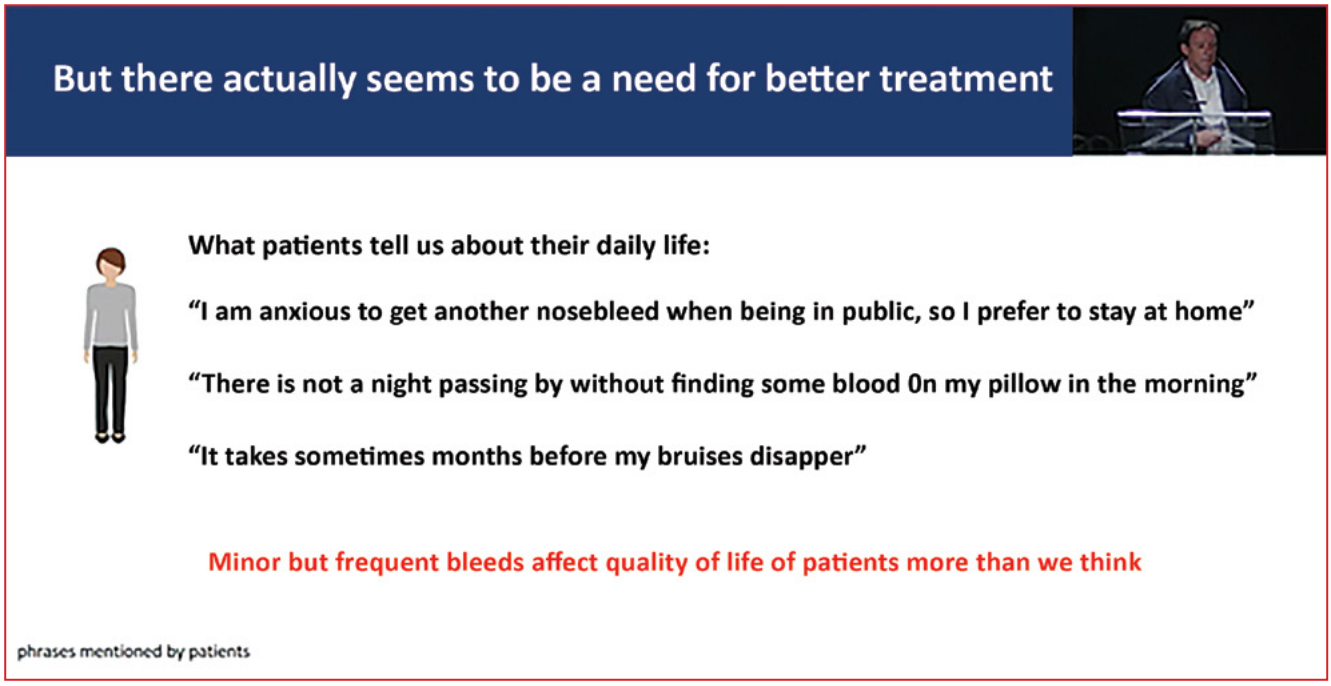
In the Arosenius Lecture medical plenary Peter Lenting explored some of the treatment issues for people with von Willebrand disease (VWD) and outlined some innovative treatments that are currently in the pipeline.

Although his VWD research is highly scientific, Peter Lenting consults his patients with VWD regularly to understand the impact of VWD and what outcomes they are seeking from treatment.

Clinicians would usually expect the biggest impact on quality of life to occur in severe forms of VWD. However, he has found there are also many issues for people with mild forms who experience frequent minor bleeds, especially women. Most patients with VWD have Type 1 (5-30% von Willebrand factor/VWF) and many are in the category now called VWF ^{low} (30-50% VWF). They have frequent minor bleeds which do bother them and affect their quality of life.

As a result, he proposed the goals of VWD treatment should be:

- To obtain a better efficacy
- To improve quality of life
- Ultimately, to cure the disease.



For his patients, this included:

- Reducing the volume that needs to be infused
- Subcutaneous (under the skin rather than into a vein) and less frequent infusions
- Gene therapy as a cure.

He outlined some current clinical trials and experimental studies and how they are contributing to these goals:

Emicizumab in Type 3 VWD

- subcutaneous, fewer infusions
- improves the half-life of factor VIII (8); might improve haemostasis (blood clotting)
- shows some promising results in early studies
- will still need to correct the VWF deficiency in some patients.

Nanobody therapy –

currently experimental mice studies

- increases factor VIII and VWF levels for at least 7 days
- corrects bleeding in VWD Type 1
- could this be used as prophylaxis in humans - a subcutaneous infusion once weekly or every 2 weeks?
- or could the nanobody molecule be used as gene therapy?

Suzanne O'Callaghan is HFA Policy Research and Education Manager.

Robyn Shoemark

Changing treatment, changes diagnosis

Chair ~ Kate Khair, Director of Research, Haemnet, London, UK

Non-factor therapy in persons with acquired hemophilia A and von Willebrand disease

~ Ming Lim, Assoc Prof, Division of Hematology and Hematologic Malignancies, University of Utah Health, Salt Lake City, USA

For patients with Type 3 VWD, standard treatment is factor VIII and von Willebrand factor (VWF). For patients who have developed an inhibitor to VWF or continue to bleed despite prophylaxis, standard treatment is plasma-derived FVIII/VWF or recombinant factor VIIa and there are few options for new treatments.

Ming Lim reported on a review of off-label use of emicizumab in 8 patients with Type 3 VWD, 3 with an inhibitor and 5 without an inhibitor with frequent bleeds. Results so far looked promising, providing haemostasis for these patients. Further studies need to be conducted to check and confirm the results.

Late-breaking Clinical Research

Chair: ~ Glenn Pierce, Vice President Medical, World Federation of Hemophilia, USA

A phase 1 sequential pharmacokinetic (PK) evaluation of octocog alfa, rurioctocog alfa pegol, and efanesoctocog alfa in severe hemophilia A

~ Annemieke Willemze, Senior Clinical Research Director, Sanofi Genzyme, Amsterdam, Netherlands

Annemieke Willemze discussed the BIVV001 (efanesoctocog alfa) trial results in the Late Breaking Clinical Research session. In question time, there was a discussion about the use of BIVV001 for patients with Type 2N and Type 3 VWD. Results have been similar to people with haemophilia A, with a weekly treatment injection and an increase in the half-life of the treatment to an average of around 43 hours. This is good news for those patients with little to look forward to on the horizon of new treatment options.

Robyn Shoemark is Clinical Nurse Consultant Haemophilia/Haematology at the Kids Factor Zone, The Children's Hospital at Westmead, Sydney, NSW.

AHCDO John Lloyd Clinical Excellence Grant

Andrea Johannessen

The Australian Haemophilia Centre Directors' Organisation (AHCDO) Executive Committee are pleased to announce that the successful application for the AHCDO John Lloyd Clinical Excellence Grant 2022 round is Dr Radha Ramanan for the project titled **Phenotypic characterisation and molecular profiling of congenital fibrinogen disorders: the Australian experience**.

Dr Radha Ramanan

Dr Radha Ramanan is a Haematology Fellow at Alfred Health in Melbourne and is also undertaking a PhD at Monash University, having completed her advanced training in clinical and laboratory haematology in 2021 at Alfred Health. Radha is also the current AHCDO Research Fellow. Radha has an interest in bleeding disorders and molecular pathology and seeks to broaden our understanding of these fields through research funded by AHCDO.

Congenital fibrinogen disorders are rare disorders affecting of one of the major clotting proteins of the blood, fibrinogen. These disorders are inherited which means these patients are born with the problem. This disorder causes different problems in different patients, for example, one person may experience blood clots while another may have problems with bleeding, while someone else again may have no noticeable problems at all.

Given the rarity of this disorder, we are still trying to understand out why some patients behave differently to others. Some of this difference may relate to the various gene mutations that can be seen with this condition. Genes are the genetic material which code for all the proteins in the body, including the fibrinogen clotting factor.

This project will look closely at the types of gene mutations seen in various patients with a fibrinogen disorder by genetic testing. We will also record the types of bleeding or clotting problems these patients have experienced over their lifetime, and if any of those problems arose during pregnancy or surgeries. We will assess types of treatment these patients have received in these different settings.

This project aims to provide an analysis of real-world data concerning the cohort of people with congenital fibrinogen disorders across Australia. The results obtained will inform how successful current therapeutic approaches are in the prevention of bleeding/thrombotic events. It will also broaden our understanding of the genotype-phenotype correlation present within this disorder and guide whether molecular typing at diagnosis may help us better characterise the clinical phenotype, navigate future treatment decisions, particularly regarding prophylaxis, and ascribe inheritance risk in this complex cohort of patients.

AHCDO is grateful to the following organisations for their financial support of the John Lloyd Clinical Excellence Fund - Roche, Sanofi Genzyme, Pfizer and Novo Nordisk for their support.

More information about the John Lloyd Clinical Education Fund is available from the AHCDO website - www.ahcdo.org.au.

Dr Andrea Johannessen is AHCDO Executive Officer

Prophylaxis and tolerisation in haemophilia A

Sumit Parikh

The International Society for Thrombosis and Haemostasis (ISTH) reconvened for their annual scientific meeting this year in July in London, UK. It was the first ISTH Congress with an in-person component since 2019. There was renewed enthusiasm among the health professionals to present and share their ground-breaking research and latest developments. Australian Haemophilia Centre Directors' Organisation (AHCDO) presented one abstract this year - ***Current landscape of prophylaxis and tolerisation in patients with haemophilia A in Australia***. This was drawn from Australian Bleeding Disorders Registry (ABDR) data.

Prescribing prophylaxis is a well-established standard of care in patients with haemophilia A in Australia. Emerging trends and availability of various factor VIII (8) concentrates, including Standard Half-Life (SHL) and Extended Half-Life (EHL), and non-factor replacement (emicizumab) therapies provided an opportunity to assess the current landscape of prophylaxis and tolerisation in Australia. The main aims of this study were to review current practice in prescribing prophylaxis and tolerisation among haemophilia A patients in Australia and observe the trend over the last 7 years.

One of the most record-breaking outcomes was that 87.1% of severe haemophilia A patients and 35.7% of moderate haemophilia A patients are currently on prophylaxis in Australia. This demonstrates a rising trend in the proportion of patients prescribed prophylaxis over the last 7 years, when the proportions were 81.8% for severe haemophilia A patients and 25.1% for moderate haemophilia A patients in 2015. A significant proportion (76.2%) of children and young adolescents with severe haemophilia A on prophylaxis are prescribed emicizumab compared to 48.1% of severe haemophilia A adults (> 18 years).

With the introduction of emicizumab, most of the inhibitor patients have discontinued factor VIII tolerisation and have continued with emicizumab prophylaxis. >>

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The current landscape demands increased monitoring and evaluation studies to be able to determine clinical outcomes and quality of life in haemophilia A patients on various factor VIII and non-factor products. The emphasis is again on self-recording treatment and bleeds effectively in MyABDR in conjunction with joint health assessment to measure clinical outcomes. The patient perspective is very much central to understand the effectiveness of any treatment product and prescribed regimen. An important milestone in future studies would be to include quality-of-life questionnaires and responses for further evaluation and analysis.

As mentioned in one of my previous articles, MyABDR is a valuable tool to determine the relationship between prescribed treatment and clinical outcomes. With the number of different therapies and treatments available now and in future, patient self-recording treatment and bleeds is crucial to any monitoring and/or evaluation.

Stay safe - and keep recording in MyABDR!

REFERENCE

Parikh S, P'ng S, Brown S, Barnes C, Tan C, Carter T, Tran H. Current landscape of prophylaxis and tolerisation in patients with Haemophilia A in Australia [abstract]. ISTH 2022 Congress, 9-13 July 2022, London, UK. https://abstracts.isth.org/abstract/current-landscape-of-prophylaxis-and-tolerisation-in-patients-with-haemophilia-a-in-australia/.

Sumit Parikh is the AHCDO ABDR Senior Research Fellow

World Hepatitis Day 2022

World Hepatitis Day is marked globally on 28 July. This is part of a worldwide campaign to eliminate viral hepatitis by 2030. In 2022 the theme is **hep can't wait**, reminding us that we need to be proactive in our efforts.

Hepatitis C has had a profound effect on our community. In Australia many people with bleeding disorders acquired hepatitis C from their plasma-derived clotting factor treatment products or other blood products before 1993. Several safety measures were introduced by 1993 and the risk of bloodborne viruses from plasma-derived clotting factor products in Australia is now considered to be extremely low. But many people in our community live on with the consequences of those early infections.

TAKING ACTION

Highly effective hep C treatments are available in Australia. They are easy to take – one tablet a day, not injections - with cure rates above 95% and few if any side effects.

Who is at risk?

If you had clotting factor or a blood transfusion before 1993, you could be at risk for hepatitis C.

Many Australians with bleeding disorders and hepatitis C have now had treatment and been cured – but some might not even know they have hep C. You may have had very few treatments in your lifetime and never thought you would be at risk for hep C. If this is you, don't wait. Talk to your doctor about being tested – find out if you have hep C. Testing and treatment is simple. Hep C can be cured

WERE YOU CURED OF HEP C?

Did you have liver damage or cirrhosis?

Has your liver recovered from hep C?

Don't wait to find out.

Call your hepatitis doctor or your GP to check your liver test results. Find out whether you need ongoing follow-up with a liver specialist.

REMEMBER

If you had cirrhosis or extensive scarring before being treated and cured of hep C, you still need to have a liver ultrasound scan every 6 months long-term.

KEEP YOUR LIVER HEALTHY

Have a balanced diet, maintain a healthy weight, and avoid or minimise alcohol intake.

Sadly, some people with bleeding disorders and hep C have very advanced liver disease caused by long term infection. Close liaison between hepatitis or liver specialists and Haemophilia Treatment Centres is very important for care and treatment. Research is continuing into new and improved hep C treatments and management of advanced liver disease.

PERSONAL STORIES

We thank John and Jake for sharing their experiences with hep C – clearing the virus or being cured and caring for their liver health. Read their stories in this issue of *National Haemophilia*.

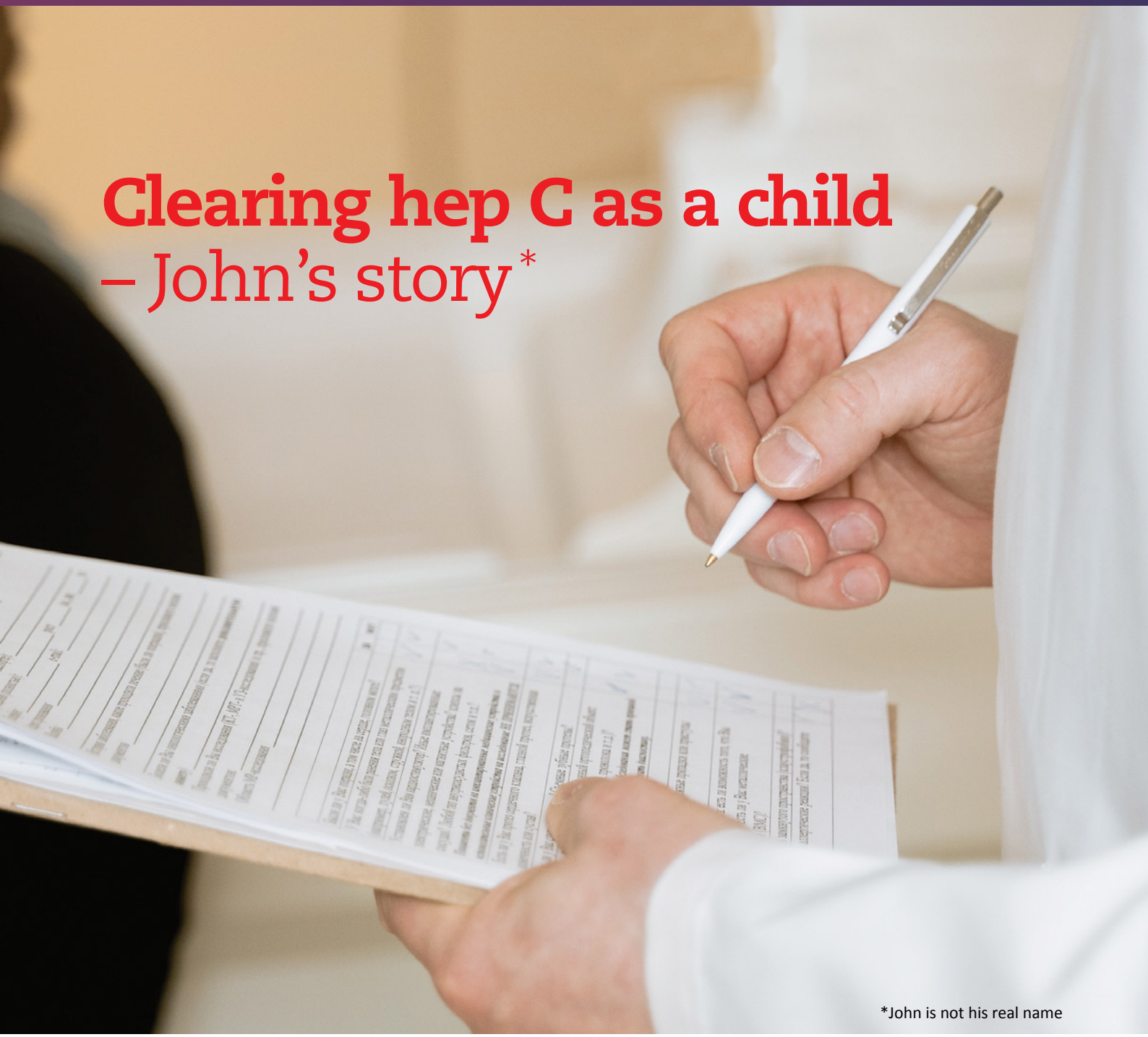
As a Partner in the national World Hepatitis Day Campaign, HFA works with Hepatitis Australia and State and Territory Foundations on the annual Australian awareness campaign and is committed to making a difference on hepatitis C in Australia.

FOR MORE INFORMATION

Visit

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

Clearing hep C as a child – John's story*



*John is not his real name

For most people who were exposed to hepatitis C through their treatment products, the diagnosis experience was more than 30 years ago. If they were diagnosed as a child, it might have been their parents who received the test results and they might not have been certain whether they still had hepatitis C until they were older.

John had hepatitis C as a child and cleared the virus naturally. He talked about his experience.

BEING DIAGNOSED

'It was in the early 1980s and not much was known about hep C at the time. I was a kid, around 9 or 10 years old, and when I was passing dark brown urine like tea. My parents thought that wasn't right and sent me off to hospital. They ran tests and diagnosed that I had what was called non-A non-B hepatitis.

'I lost heaps of weight during that time and was very jaundiced and yellow. I can't remember how long I was crook for, but I was in hospital for two or three weeks. After that I had to get back up to strength and put weight back on and try to get back to normal.'

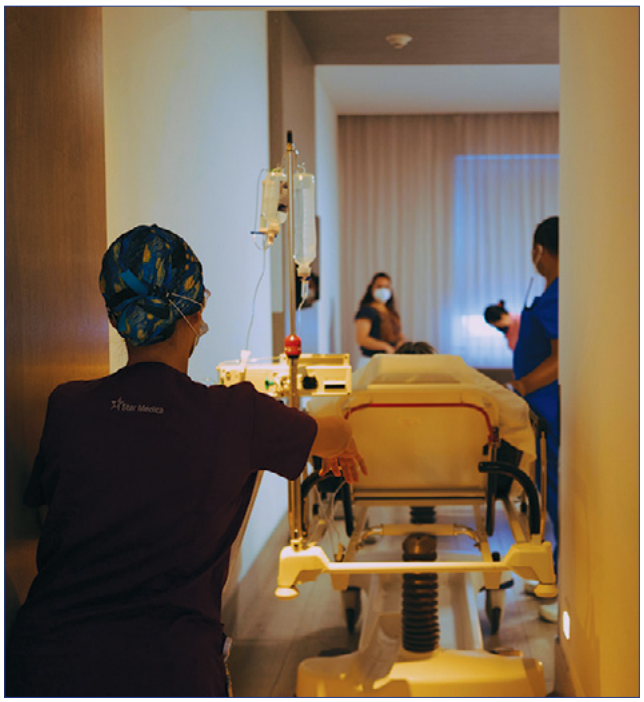
Although John and his parents didn't speak much about it over the years, he found out later that his time in hospital was traumatic for all of them.

'I recall it being a bit like COVID times. I was in isolation in my own room and wasn't really allowed visitors. My room had a glass window and my parents could only wave to me or speak to me from the outside of the window. The nurses and doctors were masked up as well.

'It was very daunting and scary as a kid. I was so young and didn't know if I was going to survive the whole ordeal. And I still have mixed emotions when I think about how I obtained the virus through my blood products. You take a certain treatment for an illness that you think is going to save your life and then you end up with another life-threatening virus or condition because of it. But I am probably one of the lucky ones. Other people got HIV. I cleared hep C naturally and I didn't get HIV as well.'

Many years later, when John was celebrating a personal milestone, his normally stoic father was overwhelmed by the contrast between the happy occasion and the memory of John's hepatitis C scare and broke down.

'He said he wasn't sure I was going to survive,' said John.



CLEARING THE VIRUS

Although John isn't sure when he cleared the virus, he has had several occasions where his HCV PCR negative tests have confirmed that he no longer has the virus in his bloodstream.

'I had to obtain a few doctor's reports for one reason or another and they specified that I was diagnosed with hep C in my childhood but had cleared the virus spontaneously and this was verified in blood tests.'

Among the tests for hep C was preparation for IVF, when John and his wife were undertaking pre-genetic diagnosis for haemophilia before becoming pregnant. John commented that the IVF doctors identified that he had been exposed to hepatitis C but could also see that he had cleared the virus.

WHAT DO THE TESTS MEAN?

There are two types of blood tests to diagnose hep C.

Hep C antibody test

Positive result - shows you have had a hepatitis C infection at some stage in your life but not whether you still have hep C.

Negative result - shows you have never been exposed to hep C.

Hep C RNA test

This test is carried out if you have a positive hep C antibody test.

Positive result - shows you still have hepatitis C.

Negative result - shows you no longer have hepatitis C.

For more information, visit *Testing for hep C* (Hepatitis Australia) - https://tinyurl.com/HA-HCV-testing

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STIGMA

Stigma relating to hep C remains an issue for many affected people with bleeding disorders. For John, there is often no good reason to tell other people that he had hep C as a child:

'I cleared it naturally. it's a thing in the past and doesn't affect my day-to-day life anymore.'

However, he is conscious that he has told very few people in his life – only personal relationships and very close family and family friends - because of the stigma. This was influenced greatly by his experiences as a child.

'I was in primary school when it all came out in the news that haemophiliacs had been infected with HIV or hep C through blood products. Obviously, my classmates knew I had haemophilia and there was the stigma of them thinking do I have HIV or hep C. I didn't disclose having hep C at the time to my classmates. It was a bit daunting and I was worried whether I would be treated as an outcast, and by their parents as well. So that wasn't a very pleasant time. I kept it to myself.

'My parents probably told a few close friends but that was it. It was never spoken about outside that circle. And I thought their friends were a bit stand-offish towards me after that. The stigma is a big thing, even in this day and age.'

LIVER HEALTH CHECKS

Keeping on top of his liver health is something that John takes seriously. There are several factors that contribute to liver health, not just hepatitis C – for example, being overweight or drinking a lot of alcohol or other health conditions. And even those who have cleared hep C will need ongoing liver health monitoring if they have already developed more severe liver scarring like cirrhosis.

John encouraged others to have any tests that are recommended for them individually and to have hep C treatment if they haven't already.

'Liaise with your treating doctor and see if you need more tests. Because there are treatments now that can cure hep C in a short time if you still have the virus. Treatment has definitely come a long way from the early days.

'Keep an eye on your liver. Even though you might have been exposed to hep C 30 or 40 years ago and you might have cleared the virus, it can still have some ramifications later in life - because there are occasions where it can lead to cirrhosis or liver cancer. So it's good to keep an eye on your liver and get any tests that you need, just to make sure there are no long-term consequences.'

Turning liver health around after hep C – Jake's story*

*Jake is not his real name

'Now that my liver is working better, everything is better.'

For Jake, managing hepatitis C and taking care of his liver health has been a long journey, but every step has been worthwhile.

Jake has mild haemophilia B. He first found out he had hep C as a young boy in the 1980s when the children's hospital called him in for testing. At the time he was more worried that he might have HIV, as that was a big concern for people with haemophilia.

'It was a strange time. When they told me and my parents that I had non-A non-B hepatitis – that's what hep C was called then – the nurses were all gowned up and wearing gloves and masks. I had never seen it before. It was a bit like COVID.'

LIVER HEALTH PROBLEMS

After his diagnosis Jake was monitored regularly with liver function tests. His doctors explained that hep C might progress slowly and they would need to keep an eye on his liver health.

'For quite a few years there was little change and I didn't think much about it. Then in my mid-20s the doctors could see some small differences in my liver function tests. I had a transjugular liver biopsy and that showed a little bit of damage in my liver.'

But suddenly in his early 30s his liver health started deteriorating and at a rapid rate. This was some years before 2016, when the new highly successful DAA hep C therapies became available in Australia. He commenced triple therapy treatment with telaprevir, interferon and ribavirin but did not respond to treatment.

'That was really disappointing and I was very upset. The next year I had another biopsy and they told me that I had definitely developed cirrhosis. Things got really bad for me then. I felt horrible, tired and very anxious.'

HEP C CURE

With a combination of non-response to treatment and haemophilia, his options for clinical trials in Australia were very limited at the time. Desperate, he applied for clinical trials around the world and just as he was losing hope, he had a positive reply from a clinic in the USA. This was no ordinary situation. He persuaded the clinic he could take responsibility for himself and his health, his hospital in Australia provided all his clinical documentation, then he travelled to and from the USA to participate in the trial.

His hep C treatment was Harvoni® (ledipasvir and sofosbuvir), one of the DAA therapies that are now widely available in Australia.

'I had one tablet a day for 12 weeks. Side effects? Some confusion and blurry vision, but my liver was in pretty bad shape so it might have been something else. And it all returned to normal as soon as I finished treatment. It was a piece of cake compared to interferon.'

When he received his results showing he had been cured of hep C, Jake was not surprised.

'I already knew. I felt different. I felt good.'

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CIRRHOSIS MONITORING

Even though his hep C has been cured, Jake had cirrhosis before treatment, which means he needs ongoing liver health monitoring.

'I have to have 6-monthly liver checkups because with cirrhosis I'm at an increased risk of cancer. I go to a liver clinic at the hospital to see a specialist – the same hospital as my Haemophilia Treatment Centre, just in case there are any issues with my haemophilia.'

Jake sees this as part of his routine care. He has some tests and an ultrasound then follows up with an appointment with the specialist about a week later to discuss the results. With COVID, his specialist appointment is now via telehealth.

'It's no big deal. As we get older, we need to be more careful of our body anyway. The liver function tests are normal blood tests – I just have them every 6 months, along with other regular blood tests I have, like cholesterol and blood sugar. And I can do them at the pathology unit at the weekend if I want to. The ultrasounds are at the hospital. They are painless, take 5 minutes. And the hospital will do their best to work around your schedule.'

While it can involve time off work, Jake thought the effort was well worth it.

'I don't think anyone likes to have health checks all the time, but it is definitely peace of mind. If they find something, they can act on it straight away. You don't want to find out down the track when there is not much you can do.'

LIVER HEALTH AND WELLBEING

Since his hep C cure, Jake's liver health has improved remarkably.

'I still have a bit of liver damage now, but it is not nearly as bad as before treatment. It's amazing.

'Now that my liver is working better, everything is better. I have as much energy as when I was 21 years old. My moods are better. I don't get itchy, I don't get brain fog, I feel fine after a big meal.'

Jake has made changes to his lifestyle to take care of his health generally and thinks this has made a difference to his liver health recovery.

'I do help my body a lot. I haven't drunk alcohol in 14 years. I don't smoke. I walk a lot. I eat a Mediterranean diet and don't eat much red meat or sugar.'

His message to others?

'We are living in different times. Anyone thinking about hep C treatment, just do it. Don't hesitate. No one should live with hep C, especially these days. Treatment has never been easier.

'The damage to my liver happened so quickly. For years there was no change, then suddenly it went from bad to much worse.

'Make the effort to have treatment and follow up on your cirrhosis. Don't give up. Life's too short to take chances.'

For more information visit
Living with cirrhosis (Hepatitis Australia) -
https://tinyurl.com/HA-cirrhosis

YOUTH NEWS

Becoming a youth leader

Chris spoke to HFA about being a youth leader with his local Haemophilia Foundation, camps, catch-ups and life with haemophilia these days.

My name's Chris.
I am 21 years old and I have severe haemophilia A.

Tell us about you and your bleeding disorder

I am at uni now and also work part-time in hospitality.

I am on prophylaxis treatment and have transitioned to emicizumab from a standard replacement factor therapy. I only have to infuse once a week and it's not through my veins anymore, it's through my fatty tissue. I've noticed I have less bleeds and no more spontaneous bleeds, which is good!

If I'd had this when I was younger, my life would have been a bit different. I wouldn't have had to make so many trips to the hospital. My uni work isn't being interrupted by bleeds so much anymore.

How did you become a youth leader?

I have been a Haemophilia Foundation Victoria youth leader for a few years now. There wasn't any formal initiation. Tim from Purple Soup approached me at camp and asked me to watch out for some kids on the high ropes who might be a bit scared. I walked them through how to get across the ropes safely and encouraged them.

We wear blue shirts at Foundation activities to identify us as youth leaders.

It started from that and then kept going with later camps. Then the Foundation made youth leaders into a small group – the 'Blue Shirts'.

What does being a youth leader involve?

We wear blue shirts at Foundation activities to identify us as youth leaders. While you are at camp and wearing the blue shirt, you need to take on certain responsibilities to help out where you can. That might

Chris's story

entail looking after the kids at camp, or setting up activities, or being an assistant to camp staff, for example, explaining how to use a harness.

It's very rewarding seeing someone getting ready for the flying fox for the first time who is very scared, and then they eventually do it.

What's your favourite foundation community activity?

There's nothing quite like the camps. They are almost like a support group. You go along and you don't feel so alone. It can be very isolating sometimes, especially if you don't talk to many people who have a similar bleeding disorder to you. At camp you are with people with the same experiences and can share how you have dealt with particular situations.

Recently we haven't been able to have many catch-ups because of COVID, but we are planning to do more. We try to do team-bonding experiences, like doing an Amazing Race around the city, or just hang out together and catch up.

How do you think the youth leader program helps young people in your community?

I hope the program inspires young people to encourage and support each other and to reflect on how they approach life – could it be better? Could they be more patient with others? I guess we lead by example.

What are the personal benefits for you?

The Blue Shirts program has taught me how to be a team player and to be more optimistic. It's also very rewarding – for example, seeing someone getting ready for the flying fox for the first time who is very scared, and then they eventually do it. You feel a sense of pride and happiness in what's achieved.

We strive to develop and improve our leadership qualities - and they are also good for my resume!

What are you up to these days?

I am studying interior design and architecture at uni full-time. I also work as a waiter, which involves a lot of walking and going up and down the stairs.

I do love my video games and I like to read comics. I have been doing a lot of swimming as well, which helps me to recover from a leg injury from a couple of years ago.

Read more

Want to know more about camps or other youth activities? Speak to your local Haemophilia Foundation.

And check out the stories about camps on:

- Factored In, the HFA youth website - www.factoredin.org.au
- The HFA YouTube channel - https://tinyurl.com/HFAYouTube

Photos supplied by Chris and Haemophilia Foundation Victoria and reproduced with permission.

CALENDAR

Bleeding Disorders Awareness Month
October 2022
www.haemophilia.org.au/BDAM

World AIDS Day
1 December 2022
www.worldaidsday.org.au

World Haemophilia Day
17 April 2023
www.wfh.org/whd

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BIOMARIN | CSL BEHRING | NOVO NORDISK
PFIZER AUSTRALIA | ROCHE | SANOFI GENZYME
TAKEDA

Coming up soon!

This year we're raising awareness for bleeding disorders throughout the entire month of October.

Keep your eye on our website www.haemophilia.org.au/BDAM and social media platforms.

We'll share more information about the activities and events we have planned as we get closer to the date.

ONE COMMUNITY, MANY FACES

Bleeding Disorders Awareness Month
OCTOBER 2022

Don't forget to add it to your diary - and get your promotional items order in!