National Haemophilia
Haemophilia Foundation Australia
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THE LARGEST INTERNATIONAL MEETING FOR THE GLOBAL BLEEDING DISORDERS COMMUNITY

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Red Classic

Sydneysiders, join us for the Red Classic on Sunday 7 October 2018 to kick off Bleeding Disorders Awareness Week.

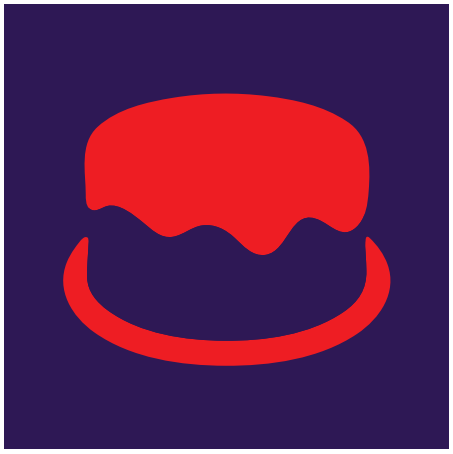
Book online at http://www.ticketebo.com.au/haemophilia-foundation-australia/red-classic.html

For more information, visit www.haemophilia.org.au/rc or contact Natashia ncoco@haemophilia.org.au or 1800 807 173.

SUN 7 OCTOBER 2018

- Leichardt Oval #3, Lilyfield (Sydney)
- 4.5km and 7km route – run or walk
- Activities hosted after the Classic
- Race starts at 9am
- Adult, child, concession and family registrations options available
- All registrations receive a free commemorative item

BLEEDING DISORDERS AWARENESS WEEK



Bleeding Disorders Awareness Week
7-13 October 2018

Red Cake Day

Bleeding Disorders Awareness Week is an opportunity for individuals and families as well as Haemophilia Foundations and other organisations to take part in a campaign and activities to raise awareness about haemophilia, von Willebrand disease and related inherited bleeding disorders throughout Australia during the week of **7- 13 October 2018**.

This year the theme is ***Living life to the full.***

What does living life to the full mean to you? Look out for the personal stories from the bleeding disorders community that we will publish during Awareness Week. We invite you to share these stories with other people you know: it's through sharing personal experiences that we can connect and increase understanding in the wider community.

More information about Bleeding Disorders Awareness Week is at https://www.haemophilia.org.au/BDAW.

RED CAKE DAY

HFA is calling on our friends and supporters to help us by taking part in Red Cake Day!

How can I get involved?

- Organise a **Red Cake Day** at your home, workplace, school, kindergarten or community group
- Order free napkins, pens, tattoos, stickers and colouring sheets and pencils to make your Red Cake Day special
- Display free posters, postcards, newsletters and raise awareness
- Tell your friends, family, colleagues about the week and encourage them to hold their own Red Cake Day event!

Once again Bendigo Banks across Australia will be supporting Red Cake Day. Pop into your local branch during the week.

To order your free promotional items, visit www.haemophilia.org.au/order

For more information

- visit www.haemophilia.org.au/redcakeday
- or call HFA on 1800 807 173
- or email Natashia ncoco@haemophilia.org.au.

Like HFA on Facebook **www.facebook.com/RedCakeDay**

Follow HFA **@Haemophilia_Au** and join the conversation at **#redcakedayhaemophilia**

Gavin Finkelstein is President, Haemophilia Foundation Australia

FROM THE PRESIDENT

Gavin Finkelstein

NEW TREATMENTS AT CONGRESS

The excitement about new and emerging treatments for haemophilia and new technologies buzzed around the WFH Congress in Glasgow. It was hard not to get swept up in the enthusiasm and many people commented that we are entering an era of change not seen for over 25 years since recombinant factors became available in the haemophilia space.

Nevertheless, we need to be cautious and we should not get too far ahead of ourselves, because many of the references were to medicines and technologies such as very novel gene therapies, which had not developed much further than an idea or proof of concept. For many of them, it will mean many years of work in laboratories, and then clinical trials before they might emerge as real contenders in the community.

ACCESS TO TREATMENT

But there are some new therapies that are very close and, as you will read elsewhere in this publication, some of these are already available in other countries, but not yet in Australia. We continue to work towards getting access for a range of treatments for our community.

The excitement of the discussions in Glasgow has led the HFA Council to another conversation about what the treatment landscape might look like in the future. Will the current patient care and treatment framework and the regulatory and funding processes in place work best with the new treatment opportunities in the offing? Will they be agile enough to assess, evaluate and measure treatment outcomes and costs, and will those processes be able to accommodate the needs and expectations of the community in the future?

The excitement of the discussions in Glasgow has led the HFA Council to another conversation about what the treatment landscape might look like in the future.

From my informal discussions with our community members, I am aware people do have expectations. I think it is important that we make sure there is not false hope and disappointment, and that we work constructively with all stakeholders to ensure accurate information is available and a clear understanding of the expectations and needs of people living with bleeding disorders and their families and carers. Our ongoing dialogue with clinicians and government decision makers aims to ensure timely access to the new treatments that our community needs and relies on solid evidence both about treatment outcomes and the impact of access on the community. This needs a robust discussion about the future we want to see and the pathways we will need to achieve that.

Sharon Caris is HFA Executive Director

REFLECTIONS ON THE CONGRESS

Sharon Caris

Each Congress I have attended has flagged new hope, and an excitement for improved treatments in the future. Congresses are full of a combination of new ideas and discussions about some of the longstanding controversies in haemophilia such as the goals of treatment, how best to describe and measure the outcomes of treatment, and debate about different aspects of the evidence that emerges. Many of the powerful personal stories shared by community representatives in presentations at Congress are a critical part of this.

I am always struck by the tension between the quest for evidence on one hand, and the implied lack of strength in the evidence of the very anecdotal and personal experiences of people living with a bleeding disorder. It is hard to measure the experiences of treatment, the benefits and outcomes that for them make such a difference to how their life plays out.

Some of the stories of people who have moved to a new prophylaxis regimen, or have treated with an extended half-life product or other novel product that is designed to stop bleeding are nothing short of amazing, and regardless of the debates about 'what the evidence says', this is also part of the evidence; the benefits for individuals are outstanding.

Just think of the difference it makes for a family with one or two young children with haemophilia where treatment with an extended half-life product can mean better cover, no breakthrough bleeds, with one or two infusions less per child per week. Or the teenager with a long history of inhibitors who has missed huge chunks of school time because of bleeds but who no longer has bleeds and only needs treatment once every month. His mother contemplates returning to work after ten years of being home to look after him, and responding to calls from his school to come and get him because he is having a bleed and is unwell. Or the middle-aged man who now treats just once a week, or even less; he doesn't have bleeds any more, and can actually use his annual leave from work to have a break with his family, instead of using all his sick leave, personal leave and annual leave, as well as extra unpaid leave because he has to stay home from work because of his bleeds. He might even dare to take on more responsibility and challenge at work. Or think about the older man who needed to go to hospital because he could no longer manage to treat himself. His arthritic joints and damaged veins had made it really difficult for him, and for his partner, but with a novel treatment his bleeds and pain were reduced considerably.

We hear of so many of these stories. They are at the very personal end of the long development of medicines from just an idea right through to observing and seeing how they work in real people and in real people's lives. The personal experience of the people using these treatment products cannot be underestimated. They are all about the quality of life a person can hope for and attain. People with bleeding disorders know this; so do their families and carers; and their clinicians. It should not be regarded as soft evidence. We must factor in the real life outcomes for them as part of the overall evidence that is accepted by all stakeholders when they are evaluating treatment outcomes.

Sharon Caris was funded by HFA to attend the World Congress.

WFH 2018 WORLD CONGRESS

1

The World Federation of Hemophilia 2018 World Congress was held in Glasgow, Scotland from 20-24 May 2018.

The WFH World Congress is the largest international meeting for the global bleeding disorders community, and in 2018 brought together approximately 5,000 delegates from around 130 countries. Health professionals, researchers, scientists, government and industry representatives, national haemophilia organisation leaders and members of the bleeding disorders community met to learn, share, and problem-solve.

The Congress program provided an innovative and comprehensive overview on the latest developments, current healthcare issues, and the challenges ahead in the management and treatment of people with haemophilia, von Willebrand disease, rare clotting factor deficiencies, and inherited platelet disorders worldwide.

In this special Congress feature, Gavin Finkelstein, HFA President and some of the HFA staff and haemophilia health professionals who attended give a snapshot of some key sessions in their interest areas.

2

The WFH World Congress is the largest international meeting for the global bleeding disorders community, and in 2018 brought together approximately 5,000 delegates from around 130 countries.

CONGRESS IMPRESSIONS

'Attending the WFH Congress in Glasgow was a really wonderful experience. It was a conference where I learnt so much and there were so many interesting sessions to attend. Five days of Congress passed in a flash. In some of the limited spare time it was wonderful to see the beautiful old city of Glasgow and the amazing architecture, and we spent a happy afternoon watching the royal wedding in a local hotel. Networking with other health professionals both local and international was also a highlight.

'It is a very interesting time to be in haemophilia - there is so much research and many new treatment modalities emerging, some including treatment for those with inhibitors.'

Megan Walsh, Clinical Nurse Consultant, Ronald Sawers Haemophilia Centre, Melbourne

'The Congress flags outside the Scottish Event Centre had the message "People make Glasgow – Welcome" and I have to say, from the moment I arrived I was struck by the friendliness of the Glaswegians. Congress felt very relaxed and easygoing. There was a real sense of optimism about the new treatments, but also about the progress in new approaches to women and bleeding disorders and VWD. It was also a great opportunity to catch up with my international colleagues: so many issues are common to us all, and I learned a lot from sharing strategies and seeing what has worked for others.

'Glasgow is home to so much scientific and cultural innovation. I was so excited to see the science centres, BBC Scotland building and the lovely Victorian and Art Deco architecture I had read about. It was a terrific environment for a Congress that was all about huge steps forward.'

Suzanne O'Callaghan, Policy Research and Education Manager, HFA

1. Entrance to the Congress trade exhibition
2. L-R: Sharon Caris, Gavin Finkelstein, Hayley Coulson, Andrew Selvaggi, Megan Sarson (AHCDO) at the Congress dinner
3. A sea of delegates entering the Scottish Event Centre
4. The SEC Armadillo, venue for the plenaries

Suzanne O'Callaghan is HFA Policy Research and Education Manager

CONGRESS: AN INTERVIEW WITH GAVIN FINKELSTEIN

Gavin Finkelstein, HFA President, spoke with Suzanne O'Callaghan about his experiences at the WFH Global National Member Organisation (GNMO) Training and the World Congress.

Suzanne: Where was GNMO Training held?

GAVIN: Training was at the Golden Jubilee Conference Hotel, in a lovely setting on the banks of the river Clyde, near the Glasgow airport. We had our own areas at the hotel and it was a great way to keep everyone together away from distractions. It is a big opportunity to reconnect with old friends, meet new people, and get fresh views, but also there were long meal breaks and time in the evening so that you could sit and have a concentrated chat with people.

Suzanne: What was different about GNMO Training this year?

GAVIN: From the beginning, there was excitement about gene therapy. There was a presentation from a young English guy who had had gene therapy, who was very positive about his experience and happy to discuss it afterwards. He talked about what the particular type of gene therapy really entailed – the weeks of steroid injections, and going to hospital multiple times a week for blood tests. As he pointed out, he still has factor levels that are 30-40% of normal, but even so, that has made a big difference to his life.

This is the first time you can talk about a 'cure' for haemophilia, which makes for a really different attitude. It is a bit like when the hepatitis specialists introduced the word 'cure' to hep C – the word highlights how much the health outcomes have improved. But there are still a few significant issues to deal with and the therapy needs to be fine-tuned. For example, around 30% of people with haemophilia have antibodies to the viral vector they have used for transporting the gene within the body in the clinical trials we have been hearing about, so they have to look into other vectors for the people this treatment doesn't suit.

The question now becomes how countries about the world – both well-resourced and developing - would be able to access gene therapy in an equitable way. We discussed this in advocacy workshops and how it could be managed in the health system, covering the realities of health economics. It is going to be an interesting situation.

I found it quite stimulating to meet youth delegates from other countries who are very active in haemophilia awareness and peer support on social media, telling their story positively and responsibly. Two young guys in particular were very engaging as community advocates: David Braun from Brazil, who is on YouTube as David Braun, and Luke Pembroke from the UK, with his YouTube vlog, **The Clot Thickens**. The humour made it quite memorable - Luke talked about how he played soccer as a goalie, not that he was a good player! But it meant he could participate in something he loved doing. The camaraderie among the young people was good to see.

I had spent quite a bit of time talking with people from other countries at GNMO Training and getting to know them and their situation. The photos and videos in their presentations at Training also captured your interest and helped you to understand what they were describing. It's important to make these contacts when you have the opportunity, then you know them and have a good relationship to build on later in international meetings or when you are emailing each other. There were delegates from more than 130 countries and it was an opportunity to get to know a large number of people well in a way that you can't in Congress.

1. The Clot Thickens YouTube channel
2. Gavin entering the Scottish Event Centre
3. Prof Alok Srivastava explaining the emergence of gene therapy

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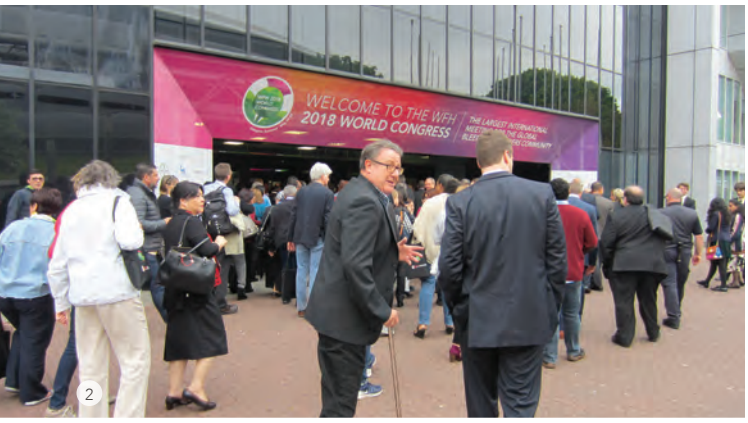

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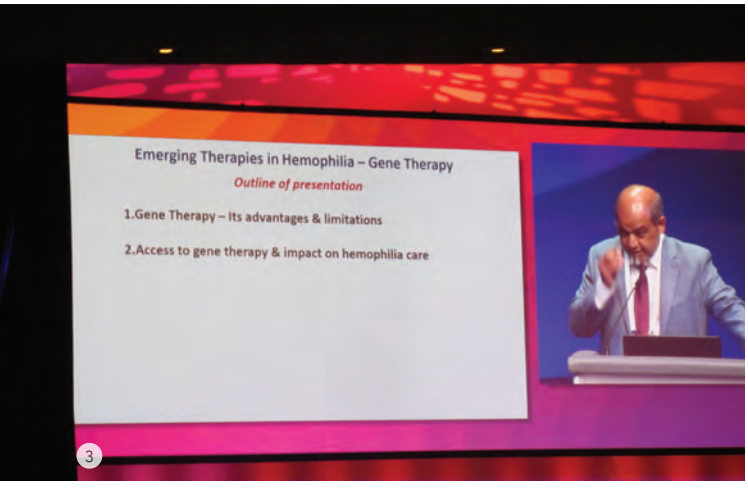

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I found it quite stimulating to meet youth delegates from other countries who are very active in haemophilia awareness and peer support on social media, telling their story positively and responsibly.

Suzanne: Did any sessions stand out for you?

GAVIN: Gene therapy was prominent, as I have mentioned. But there was a good dedicated session on VWD. I spoke in the session on education and awareness in Australia, where there was a lot of stimulating discussion. More women present with symptoms of VWD than men, and with the different symptoms and types there is a really broad spectrum of experiences, in comparison to haemophilia, where experiences can be quite similar depending on severity. It is important to be inclusive. We discussed some key messages for awareness and how to articulate them: once you are diagnosed, and have a treatment plan to manage your symptoms, you can get on and live your life; VWD doesn't define you as a person.

It is a massive issue for the developing world that VWD is rarely considered as a cause of bleeding, and this problem was also reinforced in Congress sessions. You can't have the same education and awareness campaign across the world: in some communities there are specific cultural issues and significant stigma and discrimination, which means that people are reluctant to admit that they have a bleeding problem; and some countries lack resources for diagnosis and treatment. We looked at starting with what can be done with general awareness - because VWD is more prevalent than haemophilia. Attitudes and knowledge really need to change – both among health professionals and the community.

Another issue raised was the complexities of bleeding disorders: medical presenters explained that an individual may have multiple disorders, not just a bleeding disorder, and unravelling what the medical issues are and treating them effectively is really important. We are now moving to individual treatment plans, not one size fits all.

I found the ethical issues with supplying EHLs [extended-half-life factor treatments] to resource-poor countries on a humanitarian basis very interesting. They only receive

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It was exciting to hear that gene therapy is within our grasp and achievable. We had presentations from people who are actually receiving it and it is clearly a great thing for the future.

the factor on a short-term basis. What happens when their annual treatment quota runs out and there is no more treatment product in the country? There has to be a better way to supply so that treatment can be maintained for the individual for the full year. It's a difficult thing.

I think also that we need to be vigilant about gene therapy in developing countries to make sure safety and efficacy standards don't slip – they are very vulnerable to cost-benefit approaches.

Speaking of cost-benefits, everyone is very excited about the positive impact on the improved quality of life and better health outcomes of several new products, but they present a challenge with current funding evaluation models. We want to be sure governments take into account the most important outcomes and potential long term savings. This is particularly important with new therapies like Hemlibra® (emicizumab), where we have seen very good results with inhibitors.

I noticed very few sessions about HIV and hep C now. People are doing so well with treatment that the discussion is more about lifestyle and wellbeing. It's great to see.

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

GAVIN: The emphasis on new treatments has really changed the landscape. Because this is a global community, other questions were raised, such as is there a place for low-dose prophylaxis, if this is the only treatment available. In India they have been looking at this for some years now.

You can see the impact of changing treatment approaches on the types of concerns that came up for discussion. One example: now that there is a new generation that has grown up with prophylaxis, there are new issues for people who technically have a severe bleeding disorder, but whose experience is similar to someone with a mild disorder. This involves quite significant problems, such as recognising a bleed when you have never had a bleed in your life. They need education to identify it and deal with it, rather than leaving it to somehow resolve itself. You still have a chronic medical condition and need to take it into account and need to be able to manage it through the different stages of your life.

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

GAVIN: Glasgow is a really easy place to get around, with good comfortable venues and food. Social functions were a really nice time to catch up with delegates, talk to people you know and meet new ones. There was a lot of camaraderie and everyone was so accepting and enjoying sharing experiences.

Suzanne: What were the take home messages?

GAVIN: It was exciting to hear that gene therapy is within our grasp and achievable. We had presentations from people who are actually receiving it and it is clearly a great thing for the future. News of this is getting out into the general media in the wider community; for example, there was a recent article about gene therapy in the New York Times. Obviously there are still issues about affordability and accessibility – and of course, gene therapy has to be safe and effective in the first place.

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

GAVIN: Congress brings different parts of the global bleeding disorders community together. This is a forum where we have a voice. It is an important opportunity to get the current information, to see what is happening in the world, and how other countries do things. It is a time when you engage with your peers, and learn from them.

Gavin Finkelstein was funded by HFA to attend the WFH World Congress.

Suzanne O'Callaghan is HFA Policy Research and Education Manager

WHAT'S NEW? A COMMUNITY PERSPECTIVE

Suzanne O'Callaghan

Women And Bleeding Disorders

Plenary - Women and girls with bleeding disorders: clinical and psychological issues

Claire McLintock, New Zealand

Many of us were looking forward to Claire McLintock's presentation on women and bleeding disorders, knowing that she would bring a fresh perspective, and she didn't disappoint. Her aim was to challenge the myths and taboos around women and bleeding, so as to clear the way for a new and more constructive approach to treatment and care.

She began by debunking a message about haemophilia that has been common until recently: in the words of a London medical specialist in 1886, 'It may almost be said that the females hand down the disease, while the males are the victims of it.' She had three important points to make, demonstrating each from recent research:

- Both males and females carry the haemophilia gene from one generation to the next
- For every male with haemophilia, up to 5 females in each family need to be tested
- Females with the haemophilia gene can bleed too.

Language to describe menstrual bleeding problems is changing in an effort to bring some meaning to women's experience. McLintock explained that the overarching term 'abnormal uterine bleeding' is now used in preference to a focus on specific symptoms with terms such as 'menorrhagia' (heavy periods), or 'polymenorrhea' (periods that are too frequent). Heavy periods are called 'heavy menstrual bleeding', which the International Federation of Gynecology and Obstetrics (FIGO) describes as 'excessive menstrual blood loss which interferes with a woman's physical, social, emotional and/or material quality of life'.

Her aim was to challenge the myths and taboos around women and bleeding, so as to clear the way for a new and more constructive approach to treatment and care.

McLintock then explored how the taboos associated with women and bleeding have impacted on women's experience: menstruation has been associated with pollution and in some cultures or religions women who are menstruating have been isolated or prevented from undertaking household tasks such as cooking, or from participating in religious rituals. While this is no longer practiced in most religions now, it has left a legacy of shame and embarrassment around menstruation for girls and women. In contrast, while Maoris and some indigenous tribal groups in California encouraged menstruating girls and women to isolate themselves, they saw the purpose as sacred, when women 'should not waste their time in mundane tasks or social distractions or concerns about men' but could take space, to meditate and 'to find out the purpose of life,' and devote their energies 'toward the "accumulation" of spiritual energy'.

She finished her presentation with a call to action around menstruation and women and girls; that there needs to be an approach that is positive and supportive, with concrete campaigns, such as affordable menstrual products. This work is taking place among medical specialists as well. In her final slide McLintock introduced the HOW (Haematology in Obstetrics & Women's Health) Collaborative, which aims to 'achieve excellence in health outcomes in women with blood conditions related to pregnancy, birth, gynaecological and reproductive health in Australia and New Zealand.' There is more information about this very exciting new initiative at https://tinyurl.com/HOW-collaborative.

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Voices of women: New challenges, different visions

Claire McLintock, New Zealand; Joana Baquero, USA; Karen Fahey, Canada; Paula James, Canada; Richa Mohan, USA

After some short presentations, this session launched into a lively and thoughtful discussion about the barriers to good care faced by women and girls with bleeding disorders and how to overcome them.

There was consensus that many doctors in the wider community know very little about bleeding disorders and that it can be difficult for a busy doctor to come to grips with the complexities of a bleeding disorder. In this session we heard from both women and parents of girls with bleeding disorders and health professionals (haematologists and a psychologist), which resulted in some honest and constructive tips built from their experience, such as:

- Sometimes you need to spell things out that you think are obvious. Because even the most well-meaning doctor might not get it sometimes and have no idea.
- It is complex information and that is why generalist doctors struggle to understand the information quickly.
- Records are really important form of evidence; carry them with you. But in emergency you need something brief because the doctors and nurses will not be able to read through all the records.
- It is important to quantify how much bleeding is actually taking place and using tools like the apps, menstrual charts and the Let's Talk Period self-BAT can be helpful. Also you need to document bleeding carefully, for example, are you changing your pad many times a day because it is full or for hygiene reasons.
- An effective way of educating doctors is for them to hear the personal stories of women with bleeding disorders as part of their coursework when they are medical students; hearing those stories would make a difference and they would remember it.

1. Emily Kempin at the HFA poster
2. Kate Nammacher and Suzanne at the NHF poster
3. Kathleen and her dolls

Engaging undiagnosed women and peer support

We had submitted a poster about the HFA *The Female Factors* evaluation and this provided a great opportunity to share our findings and discuss what other bleeding disorder organisations are doing with their women's programs. Kate Nammacher, an educator at National Hemophilia Foundation, USA, was nearby at the poster display. Her poster was about their *Better You Know* campaign, which uses online information and questionnaires to find undiagnosed symptomatic women and encourage them to seek testing.

The Scottish Haemophilia Society/Women's Booth in the trade exhibition offered a welcoming space to explore innovative communication methods for peer support. Kathleen Gerus-Darbison from the USA demonstrated her *Clot Not Club* project: girls and women with bleeding disorders make dolls who can then speak for them and tell their story (like a puppet). The consensus among the women at Congress was that it is a terrific idea: they thought it was very helpful as the doll could say all the things about your experiences that you don't feel comfortable saying for yourself.

He described case studies where his patients with mild haemophilia did not recognise that they had had a bleed and did not seek help from the Haemophilia Treatment Centre until some weeks later, by which time they had muscle and possible nerve damage.

Mild Haemophilia

When to assess?

Paul McLaughlin, United Kingdom

With prophylaxis treatment, gene therapy and some symptomatic women reclassified as having mild haemophilia, many more people in the bleeding disorders community now effectively live with 'mild haemophilia'.

Paul McLaughlin, a haemophilia physiotherapist at the Royal Free Hospital in London, looked at some of the musculoskeletal issues for people with mild haemophilia. Many people with mild haemophilia do not attend HTC reviews regularly or even return for their results after an MRI. He described case studies where his patients with mild haemophilia did not recognise that they had had a bleed and did not seek help from the Haemophilia Treatment Centre until some weeks later, by which time they had muscle and possible nerve damage. McLaughlin highlighted the importance of taking a helpful approach to these late presentations, using them as an opportunity to encourage patients to come in earlier and educating them about bleed symptoms and management.

Generally people with mild haemophilia show some subtle joint changes as they age, but it can be difficult to know what relates to bleeds and what to the normal process of ageing. McLaughlin pointed out the need for more research in this area.

While a musculoskeletal assessment will be most effective if it is done early, it needs to be part of a reciprocal relationship between the health care professional and the patient, where the patient can see the value of attending the HTC regularly, for example, learning how to manage both minor and major bleeds and improving their quality of life. New generations on prophylaxis treatment – who may not have experienced bleeds in their lifetime – may have a similar inability to recognise bleeds and know how to manage them as someone with mild haemophilia and it would be valuable to rethink treatment approaches to take this into account.

Together We Can Do More: Sticking To The Program

Chair : Susan Cutter, USA

I was impressed by a number of Congress sessions that tackled issues head-on and asked some frank and confronting questions. This session on adherence was particularly interesting.

Psychosocial strategies: Access, adherence and attitude

Susan Cutter, USA

Drawing on her nearly 20 years' experience as a haemophilia social worker at the University of Pennsylvania Medical Center, Susan Cutter looked closely at attitudes to adherence and the impact of new extended-half-life (EHL) treatments. She noted that non-adherence to treatment is common in chronic illness, with studies showing that individuals with chronic illness adhere to their medical treatment only 50% of the time. However, to avoid bleeds and negative outcomes, a person with haemophilia needs to adhere to their treatment more than 80% of the time.

EHL treatments have created new challenges, with more complex medication schedules and older people reluctant to give up their existing prophylaxis ('If it's not broken, don't fix it!'). Nevertheless, the annual bleeding rate is lower with EHLs at each level of adherence.

It is well-recognised that adherence decreases in teenagers, while at the same time risk-taking and the desire to be like their peers increases. Understanding what motivates them to adhere to their treatment is crucial. Recent studies have shown that they are more likely to adhere if they:

- Understand and accept their haemophilia diagnosis and treatment
- See treatment as a way of avoiding pain and preventing symptoms and arthropathy
- Have mastered self-infusion skills and have confidently established treatment routines/habits.

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He was determined to continue, and as his fitness increased, his quality of life was never better. He became a full-time personal trainer and reduced his bleeds.

Cutter's adolescent focus groups also suggested that motivation could be fostered by:

- Teasing out benefits for them personally ('what's in it for me?')
- Considering financial incentives (but they need to be adherent to be in the pool for rewards)
- Group/team activities for adolescents with haemophilia
- Encourage habits – adherence is more influenced by habit than intention
- Present it as an opportunity to serve as a role model/ leader.

Benefits vs. can I be bothered?

Lyndsay Hughes, United Kingdom

Following the lead of Cutter's presentation, Lyndsay Hughes looked more closely at the psychology of adherence. She noted that there are two different types of non-adherence, but both can occur with the same person:

Unintentional non-adherence (doses are usually forgotten or taken incorrectly by accident) - forgetting dose, infusing wrong dose, difficult venous access, failing to plan to ensure enough factor is available.

Intentional non-adherence (a decision is made not to take all of the doses as prescribed) - skipping doses, not infusing at the correct time, relying on on-demand treatment.

Difficulties with administration is associated with worse adherence, and can involve difficult venous access, pain, lack of time, or inconvenience. Older adults also have the lowest rates of non-adherence – a finding that was also noted in the Australian AHCDO prophylaxis study (reported in the December 2017 issue of *National Haemophilia*), where non-adherence peaked in the 30-39 year age group. Hughes speculated that this might be related to the change from on-demand to prophylaxis treatment.

Ironically what appears to be 'common sense' to someone on treatment could undermine their adherence. Hughes pointed to perceptions such as thinking that treatment doesn't make much difference; or that they don't need to keep taking factor if they feel like their bleeding is under control; or that haemophilia doesn't really affect them anymore. Appropriate education is clearly required, but understanding and working with their personal goals is equally important. Hughes also suggested using personalised medicine to link outcomes with their behaviour, for example, using PK (pharmacokinetic) graphs to identify when infusions should be timed.

The patient's preference

Andrew Selvaggi, Australia

Andrew Selvaggi, a well-known community advocate and personal trainer from Melbourne, was the final speaker and gave some insight into how things work from the patient point of view. Diagnosed with inhibitors at two years of age, Andrew spent his youth in a wheelchair, overweight and in a great deal of pain. When he moved to the adult HTC, he decided to make radical changes to his health and fitness and started exercising on an exercise bike, and put together an individualised program of resistance and cardio training with his HTC, along with a new treatment plan. This was an extremely challenging time for him, his family and his medical team, when exercising meant more pain and bleeding. He was determined to continue, and as his fitness increased, his quality of life was never better. He became a full-time personal trainer and reduced his bleeds.

WFH 2018 WORLD CONGRESS
Glasgow, Scotland · May 20-24

1. Andrew Selvaggi and his wife Trish
2. Prof Alok Srivastava explaining the emergence of gene therapy

He acknowledged that the pressure he put himself under made him a 'challenging patient' and that at times he pushed himself a little too hard. As a result he had an ankle fusion and knee replacement. In another presentation later in the Congress Andrew explained more about his personal journey – that his experiences with orthopaedic surgery led him to accept his physical reality and to make further changes in his life so he could have a longer-term future with his fitness. Now he has moved from being a full-time physical trainer to helping others with bleeding disorders in their sports and fitness programs; but is still training himself.

What is most important to Andrew for adhering to his treatment? His medical team had to know what motivated him and would keep him going. He wanted to keep up with school, and go out with his friends; and now is looking forward to continuing his work in sports and fitness. His message to HTC teams about motivating their patients? Find out what they want out of life, and what would make them live life to the fullest.

Gene Therapy

Plenary - Hemophilia Gene Therapy: From Trailblazer to Gamechanger

Thierry van den Driessche, Belgium

Gene therapy was a hot topic at Congress and Professor van den Driessche's presentation was an extraordinary effort to make very complicated science accessible to the audience. His main message: 'Gene therapy for haemophilia holds great potential but issues remain.'

Firstly, he noted that gene therapy is restricted to the patient who undergoes it; it doesn't affect the sperm or oocyte and so any children the patient has after treatment will still inherit haemophilia by the usual pattern.

He explained the attractiveness of using of viral vectors such as AAV (adenovirus-associated viral vector) to transport the new gene into the body: they are very efficient and there is no chance of viral infection in the way the viral vector is used. Clinical trials have now begun to establish the efficacy of these vectors in treating factor VIII and IX deficiency (haemophilia A and B), but how long the response will be sustained is still a question-mark – will it be life-long? - and the longevity of the response will be under investigation for some years to come. There is also the issue of pre-existing immunity to AAV in many people with haemophilia. Will this treatment work in this group, or be applicable to children or people with inhibitors? The immune response is also a safety issue with gene therapy using AAVs and currently steroids are used to stabilise the immune response. What is needed is a 'a stealth-style AAV' to suppress the immune response, said van den Driessche, using an image of a stealth bomber as a tongue-in-cheek metaphor to illustrate his point.

Gene therapy was a hot topic at Congress and Professor van den Driessche's presentation was an extraordinary effort to make very complicated science accessible to the audience. His main message: 'Gene therapy for haemophilia holds great potential but issues remain.'

What is in the pipeline for gene therapy? There are at least 6 clinical trials of gene therapy for factor VIII at the moment, using a range of transporters, commented van den Driessche. He went on to discuss the studies of lentiviral vectors, a promising technology, which has the potential to sustain factor expression long-term since these therapies are directed at the liver and pre-existing immunity is not an issue.

In a way van den Driessche's presentation characterised the 2018 World Congress: incredibly hopeful, excited, but cautious – the world of haemophilia treatment is changing enormously but we still need to understand how this will play out in the real world.

Suzanne O'Callaghan was funded by HFA to attend the Congress.

CONGRESS A NURSING PERSPECTIVE

The WFH 2018 World Congress in Glasgow was attended by 11 haemophilia specialist nurses from Australia. This included a Haemophilia Foundation Australia education grant, CSL Behring's Dawn Thorp Award, and The Royal Children's Hospital, Melbourne Continuing Professional Development Fund for Allied Health and Nursing Staff, as well as funding to several nurses from CSL Behring, NovoNordisk, Pfizer and Shire. This support to attend provides opportunities for Australian nurses to learn about new advances in treatment and care and to bring these back to their clinical settings. The impact this can have on practice change to benefit patients and families along with networking opportunities is invaluable.

Having attended a number of international congresses over the past few years, we noticed a heightened level of excitement with presentations of new treatments that would improve outcomes and the quality of life of patients and families. It is going to be a difficult task to remain patient for these products to be approved and accessible but hopefully they will be made available as soon as practical.

Specialist haemophilia nurses from around Australia have provided the following points of interest from sessions attended. Please do not hesitate to contact your own Haemophilia Treatment Centre if you have any specific questions.

A Longer Acting Factor VIII In Development

Janine Furmedge
Haemophilia Nurse Coordinator, Henry Ekert Haemophilia Treatment Centre, The Royal Children's Hospital, Melbourne

BIVV001 – a novel, weekly dosing, VWF-independent, extended half-life FVIII therapy: first-in-human safety, tolerability, and pharmacokinetics

Joachim Fruebis, USA

Research continues to be directed towards extending the half-life (how long factor stays in the body) of factor VIII (8) and factor IX (9). Current Extended Half-Life products (EHLs) can increase the half-life of factor IX by up to 5 times. However the increase in half-life for factor VIII products has been less pronounced (approximately 1.5 times) meaning it still needs to be given approximately twice per week. In human plasma, most factor VIII is paired with von Willebrand factor and one of the challenges in extending the time factor VIII lasts in the circulation has been its dependence on von Willebrand factor.

Joachim Fruebis, Senior Vice President of Development at Bioverativ, presented early study results of a new recombinant Factor VIII called BIVV001 in a 'late-breaking session'. BIVV001 fuses four different proteins together, including factor VIII and von Willebrand factor and reportedly makes a product that is more stable and with a longer half-life than current factor VIII EHLs.

A preliminary study (Phase 1/2a) has recently commenced in the USA looking at safety and half-life of BIVV001 in adults with severe haemophilia A. Participants receive a single intravenous dose of standard factor VIII and 4 days later a low dose of BIVV001. Blood tests are taken to measure the half-life of both products and to also test for FVIII inhibitor after 14 and 28 days.

Fruebis presented results from the first 4 participants:

- A single, low dose of BIVV001 extended the half-life of factor VIII to 37 hours, a substantial increase compared to the 13 hours seen in the standard recombinant factor VIII
- Five days after the dose of BIVV001 the average factor VIII level was 13%, and after seven days was 5.6% (factor VIII level after standard products would be expected to be 0% after 5 and 7 days)
- BIVV001 was generally well tolerated and with no development of inhibitors
- Based on these results there is the potential for once weekly or longer dosing.

BIVV001 has shown promising results in early studies. New products such as BIVV001 are subject to rigorous further study before establishing they are safe and effective and ultimately becoming available for use.

Australian nurses at Congress,
Photo: Robyn Shoemark

Childhood Obesity And Haemophilia

Jaime Chase
Haematology Clinical Nurse Specialist, John Hunter Children's Hospital, NSW

Obesity

Kuixing Li, China

As part of my experience at the WFH 2018 World Congress, I was privileged to attend many thought-provoking and practice-changing sessions. One of these was about the effect of childhood obesity on the child with haemophilia.

Obesity is abnormal or excessive fat accumulation that can or will affect a child's or adolescent's overall health. Childhood obesity is a medical condition that affects children and teenagers.

A healthy weight and height is very important for children and adolescents with haemophilia (just as it is for adults). When children and adolescents are not within a healthy height to weight ratio, this can negatively affect their healthy development. Children and adolescents with haemophilia who are obese or weigh well above their age group are at a very high risk of developing cardiovascular issues and arthropathy later in life. This can have a profound effect on the person with haemophilia as they move through their life journey.

The question is asked - why are children and adolescents obese or in an unhealthy weight range? A simple answer would be eating too much and not exercising enough. This is a very simple way of looking at an issue that is affecting the whole of the world's population of children and adolescents- not just children with haemophilia. There are very complex and interacting systems of factors within our society that contribute to this issue. These include access to calorie rich foods, increased portion sizes and increased fast food availability. These trends also include a decrease in outdoor play and sport. There is also the increased dependence on electronic devices.

What can parents and caregivers do to encourage healthy weight gain and maximise joint health through childhood, adolescence and beyond? There is a simple phrase to remember: LIVE 5, 2, 1 and 0.

5 - Have five serves of vegetables and fruit per day.
2 - No more than two hours of screen time a day
1 - One hour of physical activity per day.
0 - No sugary drinks.

There are many websites and programs available if you think your child or adolescent is overweight or obese. Talk to your HTC if you have concerns - your treating team will be able to provide referrals to appropriate services and further information if required.

Prosthetic Joint Infection In Patients With Haemophilia

Andrew Atkins
Nurse Consultant, SA Adult Haemophilia Treatment Centre, Royal Adelaide Hospital

Prevention of late infection of prosthetic joints in patients with hemophilia

James Luck, USA

The main cause of failure of a total joint replacement (TJR) is possibly infection. Prosthetic joint infection is much more common in people with haemophilia, and is a very serious complication requiring multiple operations, with prolonged disability and discomfort.

A literature review of infection in haemophilia TJR surgeries showed an average rate of infection of 8.6% in over 550 surgeries after an average of 7.6 years follow-up, with no difference in rates between HIV- and HIV+ patients. This compared to an average rate of infection in the general population of 1.5%.

The presenter recapped his own research from 1975-2001 of 90 TKRs in the US where there were reported joint infections in 16% of cases. It was posed that the joint infections were due to the introduction of pathogens through self-injection of factor in the weeks and months following surgery, and this prompted a prospective 12 year study (2005-2017) of 49 TJRs. Patients were advised of the high rate of infections in haemophilia TJR surgeries, and of the suspected cause being self-injecting. They were given education/training in self-injecting pre-operatively by HTC nurses, and reminded at every post-operative visit. Results: zero primary (unknown source) infections, with 2 secondary (known source) infections.

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1. The SSE Hydro arena Photo: Hayley Coulson
2. The Scottish Event Centre, venue for the World Congress
3. BBC Scotland, Glasgow
4. Highland Pipers outside the Congress Civic Reception

Take-home message: Vigilance in maintaining good practice is always needed when self-injecting, but especially so after major surgery, and even long after the post-operative period. Pre-operative reinforcement of technique may well be worthwhile, even after a life-time of self-injecting.

Rare Bleeding Disorders

Megan Walsh
Clinical Nurse Consultant, Ronald Sawers Haemophilia Centre, Melbourne

Overview of clinical assessment and diagnosis

Flora Peyvandi, Italy

Rare bleeding disorders have always been group where there is sparse information: numbers of patients affected are low and bleeding symptoms are slightly different and vary depending on what specific factor is missing or reduced.

Rare bleeding disorders include factor VII (7), factor X (10), factor XI (11), factor XIII (13), factor II (2), factor V (5) and fibrinogen deficiencies.

When I saw Professor Flora Peyvandi, a world expert in rare bleeding disorders, was presenting a session, I was keen to attend.

She explained that the rarity of rare bleeding disorders limited proper randomised studies into individual disorders. Treatment is often based on expert consensus rather than evidence-based guidelines. She gave some insight into treatment regimes. Rare bleeding disorders are sometimes difficult to diagnose due to technical limitations in laboratory testing.

Usually both men and women are equally affected, i.e. there is no sex-linked inheritance and the inheritance pattern is usually recessive.

Prof Peyvandi explained the bleeding pattern in rare bleeding disorders differs from haemophilia: bleeding problems are often seen at labour and delivery in affected women and also during menstruation and pregnancy, and bleeding is often seen after circumcision in affected boys, or umbilical stump bleeding after birth in male and female newborns. Bleeding often happens at time of invasive procedures and bleeding is common from mucosal surfaces, e.g., mouth, gut, vagina, etc.

The good news is there are now 22 countries contributing data to a rare bleeding disorder network on the treatment of rare bleeding disorders. So some guidelines for treatment can be formulated that will be evidence-based.

Gene mutations that cause specific rare bleeding disorders are now starting to be identified, which will assist in diagnosis and genetic counselling of families.

Currently there are only specific concentrates to treat fibrinogen, factor VII, factor XI and factor XIII deficiencies, with the majority of these still being plasma-derived. The only recombinant concentrates are for factor VII and factor XIII deficiencies but are very expensive and not available to the majority of patients worldwide.

Disappointingly there are still no available specific factor concentrates for patients with factor V or factor II deficiencies.

There has been some work on gene therapy for factor VII in animals which has been published recently.

Some of the new novel therapies in the pipeline for treatment in haemophilia may also be of use in treating rare bleeding disorders if their safety and efficacy are established in clinical trials. These include extended half-life factor VIIa; and also gene therapy, monoclonal antibodies (anti-TFPI), and small interference RNAi (siRNA) because they have an effect on the coagulation pathway rather than being specific factor replacements. These are already in clinical trial in Australia for haemophilia. If successful, they may then be trialled in rare bleeding disorders. This may result in breakthroughs for treatment of patients with rare bleeding disorders in the future, which is exciting.

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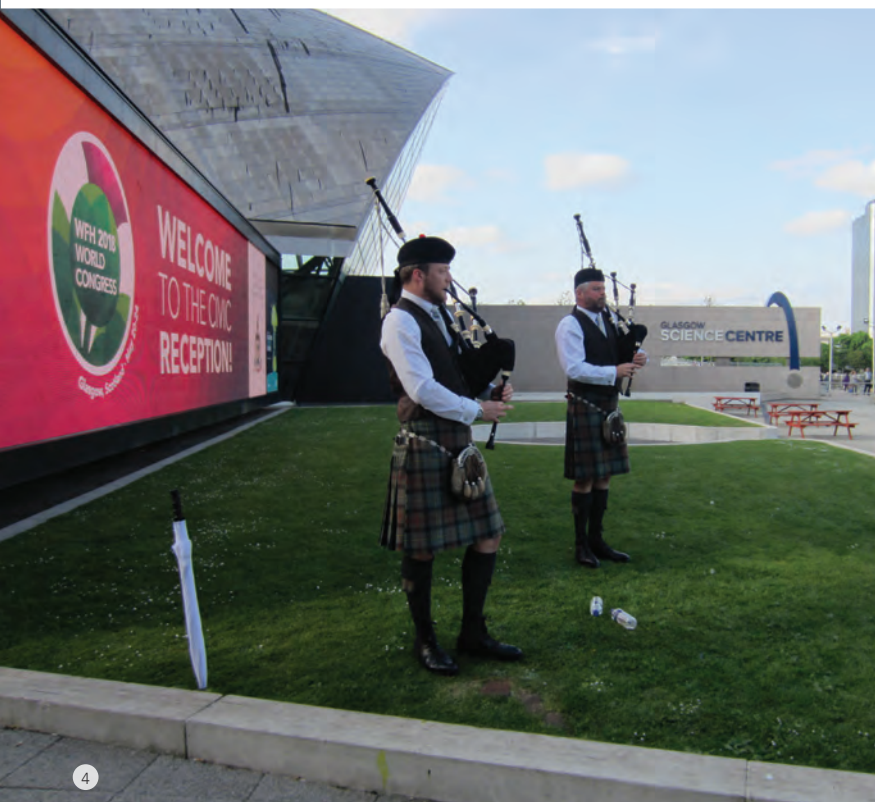

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Pain In People With Haemophilia

Natalie Gamble–Williams
Haematology Clinic Nurse, Perth Children's Hospital

Pain

Greta Mulders, The Netherlands

One of the many sessions I attended that I found interesting was the session, 'Pain in people with haemophilia', which also gave me some insight into how I can better incorporate a structured way to address pain in my patients with haemophilia A and B.

The Global Haemophilia Nurses Support Committee (GHNSC) undertook the Snap-shot Pain Survey study to establish a baseline knowledge of pain experience, investigate the annualized pain ratio and determine the efficacy of pain therapies in patients with haemophilia A and B. 53 nurses and 24 Haemophilia Treatment Centres participated. Questionnaires about pain, medications and other methods for pain relief in the last 28 days were given to patients.

There were some interesting results from the study, such as that people with haemophilia in western countries have greater pain than those from the rest of the world. Could this be because of the activities of patients in the western countries? Yet patients from western countries have better access to prophylaxis, so how can this be?

Patients with inhibitors had greater pain than those without and there was no difference in pain experience for patients on prophylaxis versus on-demand.

Lastly, they found that patients using pain medications at least once a week had greater pain than those who used pain medications on a yearly basis.

From this study we can conclude that pain management in haemophilia patients is a global issue and we need a structured way to address pain. We need to focus on both acute pain with bleeds, as well as chronic pain and follow up with the patients. The current treatment strategies for pain in haemophilia patients seems inadequate and haemophilia patients have pain regardless of treatment regime, age, or ethnicity.

As nurses we should take all these factors into consideration and advocate for our patients, involving the appropriate members of our medical team to address pain issues in our patient cohort and provide better treatment plans and strategies to alleviate their pain. Working in the paediatric setting, it is vital that we use the correct pain assessment tools for the appropriate developmental age. Maintaining consistent pain assessments of all the patients is important for their quality of life and psychological wellbeing.

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This presentation highlighted the continued need to evaluate our programs and services to empower young people to become independent and confident in managing their own health.

VWD Clinical Conundrums

Sue Webzell,
Haemophilia Nurse, Hollywood Private Hospital Haemophilia Centre, WA

Overview: Current approaches to diagnosis and treatment

Frank Leebeek, The Netherlands

I found the session on von Willebrand disease (VWD) clinical conundrums very interesting. Leebeek described VWD as the most common inherited bleeding disorder with 0.5% to 1% of the population affected. He outlined the difficulties in type classification of VWD due to inconsistency in diagnosis, and that specific mutations can result in different outcomes of various tests and added that more research needs to be done to optimise assays. He suggested bleeding scores can be helpful but may not always correlate with the patient's VWF (von Willebrand factor) levels.

This absence of correlation is particularly apparent in patients with a low VWF level. He noted that individuals who have low levels of VWF that are between 30% - 50% can show significant bleeding related to their phenotype, so they may require treatment despite their levels being above the 30% used for VWD diagnosis. These individuals can be safely treated with DDAVP to increase their factor levels; there should be no requirement for factor replacement products.

He also discussed how VWF levels rise with increasing age and comorbidities. Interestingly, the greater the number of comorbidities a patient has, the higher their VWF levels can rise, often into levels of low to normal VWF ranges. He advised that these increases in levels do not prevent bleeding. In spite of higher VWF levels, the bleeding tendency will remain the same, and treatment is still appropriate in these patients. Treatment with DDAVP or von Willebrand factor or factor VIII is still required; however, lower doses may be needed. It is important not to treat by the VWF level but according to the patient's bleeding history.

Challenges Of Ageing, Dental Care And Health Economics

Beryl Zeissink, Clinical Nurse Consultant – Haemophilia Queensland Haemophilia Treatment Centre, Royal Brisbane & Women's Hospital

Understanding ageing – a nurse's perspective

Cathy Harrison, United Kingdom

To open her presentation on understanding ageing, Cathy Harrison quoted statistics from the UK Haemophilia Centre Directors Organisation Annual report for 2015/ 2016, which demonstrated that people with haemophilia are reaching ages of 60–75 years more so now than they did 40 years ago. Important factors for this are healthy diet, maintaining physical activity, good sleeping patterns, maintaining social networks, and looking after ones' mental health. Maintaining a healthy weight decreases the stress applied to the joints, especially knees, and this helps with bleed prevention. Good fluid intake assists with venous access. Keeping up a relationship with your primary care team, i.e. your General Practitioner (GP), enables a team approach with you and your GP to perform regular health checks including blood pressure checks, cancer screening, and diabetes screening. 1 in 3 cancer deaths are preventable with earlier detection. Your GP is in a good position to recognise red flags to maintain your physical and mental health.

Healthy ageing - getting long in the tooth

Alison Dougall, Ireland

Alison Dougall challenged the audience with the importance of oral health. She stressed that oral health is not just the absence of disease but influences many aspects of general well-being – our ability to speak, smile, smell, taste, touch, chew, swallow and convey our emotions through facial expression. You need 20 functional teeth to be able to eat.

Bleeding from the gums is a symptom of inflammatory disease, so treating with factor should not be the only management. If you have bleeding gums you should see a dentist to assist with management of gingivitis. Long-term gingivitis in the elderly can affect dentition as a result of receding gums. In the elderly haemophilia patient restriction of joint movement can also affect the quality of teeth-brushing. Also as we get older, it is more likely that individuals take more tablets. Taking many tablets can increase dry mouths in the elderly, and the saliva therefore does not protect the mouth. Broken teeth also need intervention as early as possible.

Regular visits to the dentist helps to catch not only gingivitis but prevent dental decay. In the elderly oral cancers may also be identified by a dentist. Early detection improves survival rates.

The value and cost analysis of innovative therapies: the Canadian experience

David Page, Canada

Also of interest was David Page's presentation in a session on health economics, where he spoke of the complications in Canada's health system in relation to accessing extended half-life (EHL) products. Quebec has had difficulty accessing these products with different access criteria to the remainder of Canada (9 provinces and 3 territories). Quebec has 3 patients approved for EHL, whereas the rest of Canada has 150 to 200 patients who have switched to EHLs. For me, this highlighted the importance of a national approach to funding of treatment, as we have in Australia with the National Blood Authority, to ensure equity in access to new treatments.

Transforming Transition

Anne Jackson, Nurse Consultant - Haemophilia
The Michael Rice Centre for Haematology/Oncology, Women's & Children's Hospital, Adelaide

Transforming transition toolbox

Susan Hook, United Kingdom

Susan Hook, Advanced Nurse Specialist from Edinburgh Haemophilia Centre in Scotland, presented on the topic of young people with inherited bleeding disorders transitioning to adult services in the UK. Susan identified a model of care that was reflective of the model in Australia which typically:

- transferred patients from the paediatric setting to the adult setting between the ages of 16 to 18
- the local paediatric team was responsible for preparing the young person
- the focus was on the young person becoming independent, proficient at self-treatment and developing knowledge of their bleeding disorder
- and has been carried out over many years.

Her presentation then focussed on new resources that had been identified and developed to assist and improve transition for young people. With the support from Haemnet, an online community that connects healthcare professionals who manage people with bleeding disorders, and with guidance from National Institute for Health Care Excellence (NICE), a 'Transforming Transition' nurse-led initiative was developed. This involved reviewing current practice, agreeing on outcome measures, delivering and evaluating a patient-led development program.

The initiative developed a shared understanding of the life stages and the steps leading to independence. She highlighted that documentation was seen as an indicator of effective transition practice, and the initiative developed documentation materials to assist the transition program. This included the 'Ready, Steady, Go – Hello' framework designed specifically for people 11 years and upwards, which covers knowledge of the condition, self-advocacy, and health and lifestyle. It set out a clear checklist of steps and what was to be achieved that would help design a program to suit the individual.

To assist with developing resources and identifying areas for improvement, they held a workshop with young people and a parent and identified what they thought would improve their transition to adult service. One key theme was empowerment to becoming the expert patient; and they also identified the need for further resources, including animations and educational apps. Some of these have now been developed by Haemnet including a YouTube channel with animations that can be accessed by health professionals. An educational game app for young people called *Haemic's Challenge* developed by Cardiff & Vale Local University Health Boards can be down loaded for free. This game allows the young person to explore the life of someone with haemophilia.

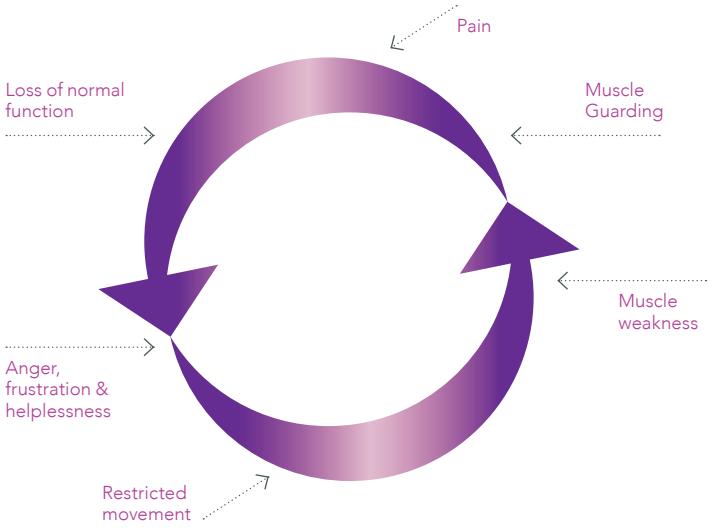
Susan concluded with the note that further work needs to be done, including piloting and evaluating the tools with small groups.

This presentation highlighted the continued need to evaluate our programs and services to empower young people to become independent and confident in managing their own health. At the Women's & Children's Hospital we have followed a transition program which highlights the transfer of knowledge and care to the individual through targeted age related milestones, but tailoring to the individual is becoming a priority. The resources developed in the 'Ready, Steady, Go – Hello' program could assist with this process as well as providing innovative online resources for education instead of the traditional educational resources we are currently using.

Hayley Coulson is Physiotherapist at the Queensland Paediatric Haemophilia Treatment Centre, Lady Cilento Children's Hospital, Brisbane

PAIN AND MUSCULOSKELETAL ISSUES

Hayley Coulson



85% of people with haemophilia had suffered pain in the last 6 months. 89% reported this interfered with their daily life. 50% indicated this was constant pain.

The World Federation of Hemophilia Congress in Glasgow provided a great opportunity to listen to speakers from across the world. This report will briefly touch on some of the sessions I attended and found most valuable as a physiotherapist.

Pain

Plenary - Gaining insight into the complexity of pain in patients with haemophilia

Nathalie Roussel, Belgium

Roussel commenced her presentation with research studies showing that there was a high prevalence of pain in people with haemophilia. 85% of people with haemophilia had suffered pain in the last 6 months. 89% reported this interfered with their daily life. 50% indicated this was constant pain.

Roussel gave a very interesting overview of how pain works and some of the issues for people with haemophilia.

Pain is an unpleasant sensory and emotional experience associated with actual or potential tissue damage or described in terms of such damage.

International Association for the study of pain

Abnormal pain physiology results in an overactive ascending pathway to the brain. This leads to critical levels of nerve excitement which can impact on the message to the brain ('action potential') and increase the pain response and decrease pain thresholds.

However, neuroplasticity (changes in the function, structure and biochemistry of the brain) can change the brain/pain cycle. Pain results in increased brain activity. Emotional responses such as stress, depression, worrying, fear of movement and hypervigilance should be considered when managing pain as a patient's behaviour will impact on their recovery. If pain is impacting on an individual's day-to-day activities, appropriate referrals and action needs to be taken to support this person in managing the pain as best as possible.

Education about pain is encouraged, and treating individuals need to be on the same page (psychologist, physiotherapist, doctors and nurses).

It is important to pay attention to the pain mechanisms and establish a proper treatment plan:

Type of pain	Treatment approach
Nociceptive pain, i.e. pain arising from the stimulation of nerve cells (acute vs chronic). In haemophilia prior cause is tissue damage.	Medications?
Neuropathic pain: nerve related pain	Medications?
Neuroplastic pain: pain is arising from the CNS (central nervous system)	Pain is not always a reliable signal. Medication?

Hayley Coulson and Moana Harlen at the Congress, Photo: Hayley Coulson

Progress Of Joint Arthropathy: Cradle To Grave

Chairs: Nicholas Goddard, Angela Forsyth

Childhood

Manuel Carcao, Canada

This was my favourite presentation of the Congress. Dr Carcao focused on the importance of early presentation and treatment in joint bleeds. He discussed the mission to preserve joints from childhood to improve joint outcomes in adulthood. Children, whether they have haemophilia or not, are born with perfect joints. He discussed three case studies of severe patients with different outcomes:

He highlighted the importance of treating early and remembering that it takes at least one week for the synovium to resorb the blood. Every bleed can potentially damage the synovium, and this holds an even greater risk if treatment is delayed. He ended the presentation with an MRI of a poorly managed ankle bleed which showed blood within the joint 5 weeks after the initial presentation.

Bad Outcome	Not a good Outcome	Good Outcome
18-year-old male.	18-year-old male.	18-year-old male.
Moved from another country where they used "on-demand" therapy. Started prophylaxis late.	Commenced prophylaxis early but had poor adherence.	Commenced prophylaxis early, excellent adherence.
Imaging reveals right elbow and right ankle damage with osteochondral (smooth surface at the end of bones) changes	Imaging reveals right knee, right ankle and left ankle changes with increased fluid and hemosiderin (a protein that stores iron in your body) deposits	Imaging showed no damage.

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Overall the Congress for 2018 was informative with lots of new research arising from HTCs around the world.

Rapid Fire Presentations

Chair: Mauricio Silva

A novel physiotherapy led musculoskeletal clinic – the perceptions of persons with haemophilia
Vishal Patel, United Kingdom

Vishal Patel is a physiotherapist in the UK who discussed a positive outcome at his Haemophilia Treatment Centre (HTC) with musculoskeletal-specific clinics. The standard multi-disciplinary clinics with a doctor, nurse, psychologist and physiotherapist run 15-30minutes and utilise the Haemophilia Joint Health Score (HJHS) and Haemophilia Activities List (HAL) as outcome measures. The musculoskeletal clinics in comparison run for 1-hour periods with the physiotherapists and utilised the HJHS, HAL and ultrasound to focus on musculoskeletal health and function with the goal to screen and prevent chronic arthropathy. As a result 95% of patients found this useful, 95% would like further musculoskeletal appointments and 87% would recommend this to other haemophilia patients.

Synovitis Re-Visited

Chairs: Adolfo Llinás, Colombia; Rachel Tiktinsky, Israel

The place of ultrasound

Carlo Martinoli, Italy

Classification and difficulties of assessment

Horacio Caviglia, Argentina

Pitfalls

Sylvia Thomas, Brazil

Ultrasound is being used more across the world in many HTCs to review joint, synovium and cartilage changes in people with bleeding disorders. For the health care practitioners who use ultrasound in HTCs, this session covered some valuable technical issues relating to diagnosing.

Many discussions throughout the Congress recommended appropriate training in use of ultrasound to ensure effective diagnosis and avoiding the synovitis "mimickers". This session discussed two mimickers in particular:

- Fat pads: At some locations, distinguishing synovium from hypoechoic fat might not be straightforward. The synovium is contained in a well-defined pouch in continuity with the joint line. Always remember that bone becomes concave when there is no cartilage, as cartilage is convex in shape.

- Intra-articular blood: In a 24 hour period blood shows a homogeneous echogenic pattern. With the ultrasound probe compression the blood shows a swirling motion and it squeezes blood away. In the later stages of a bleed, fresh clots are echogenic but do become hypoechoic with progressive lysis of the red blood cells. The best ways to distinguish blood from synovium is to review the wall thickening as blood does not attach to the walls like synovium. Blood has smooth margins opposed to the irregular margins of the synovium. Blood is also free of colour flow at doppler imaging.

Ultrasound has been utilised as an effective assessment tool that is also inexpensive when compared to other methods of imaging. Ultrasound is recommended in combination with a comprehensive physical examination.

Conclusion

Overall the Congress for 2018 was informative with lots of new research arising from HTCs around the world. While there are breakthroughs with the utilisation of ultrasound in assessment, in other areas such as general bleed management, musculoskeletal assessment and outcome measures remain the same, with recommendations made for ongoing research into the most effective outcome measures for the bleeding disorder population.

I would like to thank Haemophilia Foundation Australia for the opportunity to attend the Glasgow Congress to not only learn about emerging changes in the management of haemophilia at an international level but also the opportunity to network with the bleeding disorders community.

Hayley Coulson was funded by HFA to represent the Australian and New Zealand Physiotherapy Haemophilia Group at the WFH 2018 World Congress.

Dr Moana Harlen is the Senior Psychologist - Haemophilia at the Queensland Haemophilia Centre, Lady Cilento Children's Hospital, Brisbane

MUSIC THERAPY

Moana Harlen

Psychosocial Professional Development Day: Keeping The Energy Going

Chairs: Silvina Graña, Argentina; Tony Roberts, South Africa

The psychosocial component of the 2018 WFH Congress began on Sunday 20 May with a professional development day for the social workers and psychologists who provide services to individuals and with bleeding disorders and their families from all around the world. It's a great opportunity to be reacquainted with New Zealand colleagues who I normally meet with once a year at the Australian and New Zealand Haemophilia Social Worker and Counsellor's Group meeting in Melbourne and to network and meet new colleagues to share information and ideas.

The agenda for the day was jam-packed with a variety of interesting topics consisting of key activities the WFH Psychosocial Committee have been up to in the past few years, presentations on alternative therapies (music, mindfulness, art), development of educational materials, parenting and youth leadership programmes and a presentation from the UK psychologists and social workers discussing the processes they have used to create health professional national groups.

Music and mental health of PWH

Tim Ringgold, USA

Rather than go through all the presentations I attended at the Congress, I thought I would share in more depth a presentation on the psychosocial day that I found particularly interesting. This presentation was by music therapist Tim Ringgold from Orange, California, USA, who explained in a very dynamic and energetic way how music can heal. I am going to describe my experience of his presentation through a psychologist's eyes: I'm sure you will know that psychologists are very research and evidence-based, so I was intrigued to hear about this 'alternative' therapy and had some healthy scepticism but kept an open mind. Of course, we have called on music therapists to assist some of our children in the hospital to help calm them during venous access but I did not have any knowledge about how it worked exactly other than it was peaceful and helped to not only soothe the child but all of the adults in the room including me.

Moana Harlen and Linda Dockrill from NZ

How music therapy works

Tim's opening statement was 'you are rhythm'. I pondered about what this meant: certainly we dance, sing and clap to music, so this didn't seem a too far out there statement. He continued describing how most of us are prescribing ourselves music, for instance, when we create a playlist. We listen to music not only for pleasure and entertainment but also to exercise, relax, study, and when doing such mundane activities as housework (the only way I can get my vacuuming done!) and so forth. I found myself starting to relate to what Tim was saying so became intrigued about what else he had to share.

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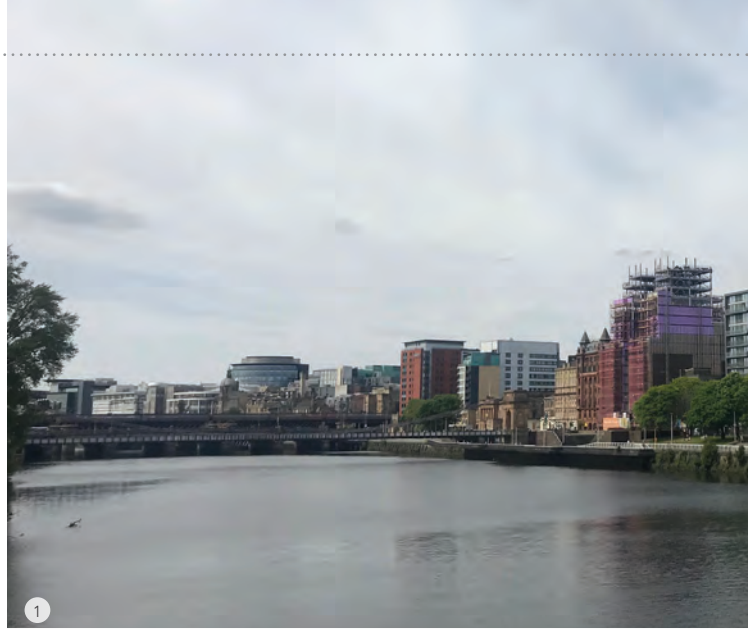


1

2

He mentioned that there is a body of evidence that suggests music therapy works - now he's talking my language. The human brain is wired for music and requires every sub-area of the brain to process, i.e., such as when processing the pitch interval and pitch pattern. This was in keeping with my own knowledge about how both sides of the brain are activated when musicians play and read music. I also really liked how Tim described 'music is heavy lifting for the brain'; probably more so because it means I can say that I worked out this week every day on the way to work. So how does it do this exactly? I was pleased to hear it does this via the reward systems in our brain (something psychologists are familiar with) which gave this information a good dose of credibility for me. Most of us are familiar with the feel-good hormones such as dopamine (not enough can cause depression) and oxytocin (bonding hormone): research has shown listening to music increases these hormones (just confirming what we already knew). Some research has shown that babies who bounce in rhythm with an adult were more likely to pick up pencils to help them out afterwards, suggesting that when babies and an adult danced in time together, they were more likely to lend a hand, so this increased social bonding.

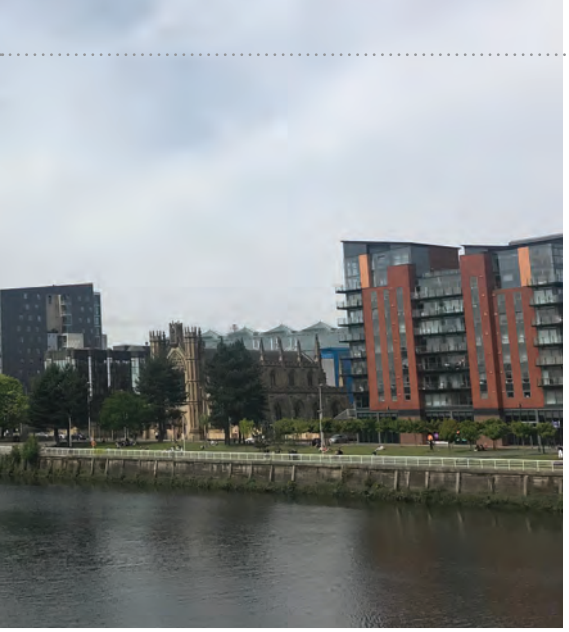
Stress management and music

Tim then discussed the role that the stress hormone cortisol plays in the stress response. This response was very beneficial for our ancestors when their stressor was a tiger that needed to be outrun (psychologists call this stress response - fight, flight, freeze); however today such stressors needing to be outrun can include a new diagnosis, having medical procedures, or learning now to access a child's veins. We know that chronic stress is not good for the immune system: the stress response was designed for acute emergency situations that were normally of a short duration, not for the stress hormones to stay in our bodies over a long period of time and thereby have negative consequences such as a decreased immune system. Additionally, a tiger was a real stressor whereas many stressors that people experience today are imagined, but when the mind experiences threat, be it imagined or real, it will activate the stress response. What tigers are you running from and are they real or imagined? The stress response can be reset (down-regulated) in a minimum of 8 seconds and you can be in charge of that.

Tim's succinctly put prescription was 'when stressed add some music have some wellness.'

Music helps by:

- Increasing present moment awareness of mind and body (similar to mindfulness)
- Increasing connection between people
- Allowing expression of emotion, for example: think about that situation that was frustrating and use shakers to shake it out or pound on a drum.



1. View across the river Clyde Photo: Hayley Coulson
2. SSE Hydro arena
3. People's Palace

The human brain is wired for music and requires every sub-area of the brain to process, i.e., such as when processing the pitch interval and pitch pattern.

- Increasing relaxation or decreasing anxiety and stress
- Increasing exercise opportunities (e.g. dancing, vacuuming with vigour).

Tim also discussed creativity as a coping skill in terms of solving problems by being creative. My initial response to this was 'oh dear I don't have a creative bone in my body'. However, he described creativity as not being in the domain of being artistic but can include everyday things such as the way you look. He noted one way you can build creativity muscles is by playing music. You can use this to help reset the stress response. If you play a rhythm that is near the heart beat rhythm you can then either up regulate (faster rhythm to stimulate and make happy) or down regulate (slower rhythm to calm and soothe).

Tim finished off the session by taking out his guitar and getting the audience to join in and sing along with him to 'Lean On Me:

Some days gotta lean on our families and friends etc
Some days you learn on me and I lean on you and we don't keep score.

Lean on me when you're not strong and I'll be your friend,
I'll help you carry on….

This was a fantastic start to the Congress. I certainly felt great and connected with everyone around me and have a newfound appreciation for the science behind music as a healing modality, which is in keeping with many of the psychology principles I use in my everyday practice. This was a pleasant and unexpected learning for me as a psychologist attending the Congress and I wanted to share it with you as we can all access music for ourselves and/or others in some way and listen to, play or create music thereby increasing our own and others sense of wellbeing.

Dr Moana Harlen was funded by HFA to represent the Australian and New Zealand Haemophilia Social Worker and Counsellor's Group at the WFH 2018 World Congress.

L-R: Sharon Caris, Emily Kempen and Suzanne O'Callaghan at the launch of the WFH Global VWD Call to Action at Congress

GLOBAL VWD CALL TO ACTION

WFH has commenced a global VWD Call to Action asking national member organisations and health care professionals 'to integrate and provide recognition of VWD and other rare bleeding disorders into their work.'

HFA has signed up to this Call to Action:

- To acknowledge the work that needs to be done
- To make a commitment to recognizing von Willebrand disease (VWD) and other rare bleeding disorders by taking action to create awareness, resources and provide support to improve the lives of those living with VWD.

HFA's specific commitment is to **participate in the development of national VWD diagnostic and clinical management guidelines**. This work will be led by the Australian Haemophilia Centre Directors' Organisation (AHCDO) and will be based on the international guidelines, which are likely to be released in 2020. Some of you may be aware of the international VWD clinical guidelines survey, which was circulated for comment in July 2018, and is one of the steps in developing the international guidelines.

WHY THE CHANGE?

You may notice that HFA is now starting to use the term **von Willebrand disease** for VWD instead of **von Willebrand** disorder. This is part of our commitment to align with the global VWD Call to Action and the development of the international clinical guidelines. We are adopting the current internationally accepted term for VWD, which is **von Willebrand disease** – and this is what you will also see on the WFH website and the international clinical guidelines.

PERSONAL STORIES

What is it like to live with a bleeding disorder?

What kinds of issues come up for parents, partners or siblings?

Visit the new **Personal Stories section** on the HFA website and hear from people from the Australian bleeding disorders community who have shared their experiences.

WORLD HEPATITIS DAY

World Hepatitis Day is marked worldwide on 28 July, and is part of a global commitment to eliminate hepatitis C by 2030.

Why Miss Out On A Hep C Cure?

New revolutionary hepatitis C treatments are now available in Australia, with very high cure rates and few, if any, side-effects.

More than 60,000 Australians have now been cured of hepatitis C. But around 170,000 are missing out.

How Does This Affect People With Bleeding Disorders?

In the 1970s and 1980s many people with bleeding disorders in Australia acquired hepatitis C from blood products for their treatment, especially plasma-derived clotting factor concentrates. Blood supply testing and viral inactivation manufacturing processes were introduced by 1993 and the risk of infection from human blood products is now extremely low.

We are pleased to hear from Haemophilia Treatment Centres that most people with bleeding disorders have now been treated and cured of their hep C.

However, there are still some people with bleeding disorders who have not yet taken up treatment - or perhaps don't even know they have hep C.

Who Is At Risk?

- If you received a blood product before 1993 – even as a baby – you could be at risk.
- Have you ever been tested for hep C?

What Can You Do To Help?

- Spread the message - if this is you, or someone you know, now is the time to do something about hepatitis C testing and treatment
- If you have a bleeding disorder and want to discuss hep C with your local doctor, show them the AHCDO/HFA GP fact sheet on hepatitis C and bleeding disorders.
- Don't miss out on a cure!

More New Treatments

A new hepatitis C treatment, Maviret®, was added to the Pharmaceutical Benefits Scheme (PBS) on 1 August 2018. The treatment course for Maviret® is 3 tablets once a day for 8 weeks and is suitable for all genotypes, including people with cirrhosis. It is a valuable additional treatment option for the small number of people who were not cured with their first round of DAA treatment.

In 2016 Australia became a world leader in access to new treatments to cure hepatitis C. These treatments are subsidised under the PBS and available via prescription by a general practitioner (GP) or specialist.

People with bleeding disorders and hepatitis C are now encouraged to talk to their Haemophilia Treatment Centre or GP to see what see what arrangements can be made to have their hep C tests and treatments locally. The **GP fact sheet on bleeding disorders and hep C** has been developed by the Australian Haemophilia Centre Directors' Organisation and HFA to support this and can be downloaded from the HFA website, under PUBLICATIONS.

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

For more information, visit the HFA World Hepatitis Day page - tinyurl.com/HFA-WHepDay-2018.

Kassy Drummond is Health Promotion Officer, Haemophilia Foundation Australia

Youth Update

Kassy Drummond

YOUTH CANOE JOURNEY

HFA has been working with the adventure therapy organisation Purple Soup, on a canoeing trip, scheduled for November this year! This trip will take place over three days, heading along the Murray River between Victoria and New South Wales. This won't be a normal youth camp. It's designed to challenge participants and build life skills. Participants will be involved with the planning of the trip and will be required to set goals around what they'd like to achieve during the program.

Thank you to everyone who took the time to apply for Youth Canoe Journey! Interviews will take place over the next few weeks. If you have any questions regarding applications please contact us at hfaust@haemophilia.org.au.

If you'd like to keep up to date with how things are going as we head towards the trip, we'll be posting a few updates on Factored In, so keep an eye out for those.

FACTORED IN

WWW.FACTOREDIN.ORG.AU

We have new content on **Factored In**!

Our new **transition** section is all about managing the changes that occur in your life as you move into adulthood. These are exciting times, so we wanted to make sure you have all the information you need to manage any challenges you might face.

While you're here I thought I'd let you know that we are rereshing **Factored In**! The layout of the site will stay the same, but in the coming months you may notice some new content being added, as well as some of the images being refreshed.

If you have any ideas about how **Factored In** could be improved you might be interested in our Youth Working Group. This group will have a part to play in deciding what new content we create for the site. If you are interested in joining the Working Group, or if you have any suggestions for new content, send us an email at factoredin@haemophilia.org.au.

YOUTH NEWS

BUILDING COMMUNITIES

One of the goals of HFA's youth program is to connect young people with bleeding disorders, so that they can have a support network of people who understand what it is like to live with a bleeding disorder, and may be able to offer support. As part of our personal stories for Factored In, we interviewed some of our young people to find out how they benefited from the youth programs:

Hamish, 24

Hamish spoke about what it was like hanging out with other young people with a bleeding disorder on camps.

'I feel like family camps are really important just to build up connections, so parents will build connections, kids will build connections. We could be a whole group of different ages, so the older kids will mentor the younger kids and give them some pointers… you know we hang out, follow each other on Facebook and just chat and keep up with each other.'

'We enjoy meeting up with other young bleeders of different ages, you know 'cause we all come bringing our different experiences and we share them and… we all just discuss how to help the community and support everybody else.'

Sam, 23

Sam told us why it was important to him to meet other young people and why he joined the Youth Lead Connect Program.

'It's very important to meet other people with bleeding disorders. It's really easy to internalise the problems we have and make them about us and think it's all about us, so it's important to externalize and meet other people going through the same thing, to understand who you are and your spot in the community.'

'I got involved in the leadership and mentoring program because I saw a Sam-sized hole in the program and I thought I'd fit right in. But it's mostly because I care greatly about our community and where it goes and I wanted to have my chance to help it get where I think it should go and give my opinions as much as I can. If this is going to be a part of me I'm going to be a part of it'

Dale, 29

Dale let us know why being a part of the HFA youth program was important to him.

'I think it's great to meet other people with bleeding disorders. They all have different experiences, and we all come across the same experiences too. I think it's just a way for us to come together and help each other out.'

'I got to about the age of 16 and decided that I wanted to branch out to other youth nationally, so yeah, I thought it was a good way to travel around Australia and meet other people you know, 'cause I'd never really left WA. It's opened my eyes to a lot of different things'

Ben, 23

Ben shared the benefits he received from being involved in the Youth Lead Connect program.

'I got involved in the HFA leadership and mentoring programs because I'd been doing a fair bit of similar stuff with the Haemophilia Foundation Victoria and I guess that puts a bit of a spotlight on me. I got asked by HFA to attend a training weekend and I went and I met all of these other people in similar situations all around the country. I loved it, we had so much fun and I'm still friends with most of the people that went.'

'Just having a community of people around you who understand the things you're going through and support you through that, it makes a world of a difference.'

The videos that go with these stories can be found on Factored In as part of our personal stories section. To view them, or to find out more information about HFA youth programs, head to www.factoredin.org.au.

CALENDAR

Bleeding Disorders Awareness Week
7-13 October 2018
Tel: 03 9885 7800
Fax: 03 9885 1800
Email: hfaust@haemophilia.org.au
www.haemophilia.org.au

World Haemophilia Day
17 April 2019
www.wfh.org/whd

19th Australian Conference on haemophilia & rare bleeding disorders
Sydney,
October 2019
www.haemophilia.org.au

WFH World Congress
Kuala Lumpur, Malaysia
14-17 June 2020
www.wfh.org

ACKNOWLEDGEMENTS

Haemophilia Foundation Australia (HFA) acknowledges funding grants received from the Australian Government Department of Health for communications and education activities undertaken by the HFA secretariat.

We thank the individuals, philanthropic trusts and companies which have made donations to support HFA programs, and the following companies that sponsor education programs, conferences or peer support programs run by the Foundation for the bleeding disorders community:

BIOVERATIV | CSL BEHRING
NOVO NORDISK | PFIZER | ROCHE | SHIRE

What's Happening in

SOUTH AUSTRALIA

HFA remains committed to supporting people in South Australia affected by bleeding disorders.

It was great to meet with members of the South Australian bleeding disorders community on 3 June 2018. Twenty-seven families came together at the Adelaide Zoo for the opportunity to meet and share their experiences. There was a craft corner that enabled the children to chat and play together and for several to meet others with a bleeding disorder like themselves for the first time. The parents seemed to value the chance to talk with one another, and everyone enjoyed the tour of the Adelaide Zoo which is home to the Wang Wang and Fu Ni, Australasia's only breeding pair of Giant Pandas.

There was also an opportunity for the families to hear about HFA's work and the activities of local Foundations in providing education, support and advocacy for bleeding disorders.

We are delighted that several people decided to meet for a business meeting on 11 August 2018 to explore whether there is interest in Haemophilia Foundation South Australia being re-established for the future.