

Bloodline

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Bloodline

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Disclaimer: The information contained in this magazine is not intended to take the place of medical advice from your GP, haematologist, or specialist. Opinions expressed are not necessarily those of HFNZ.

The purpose of this magazine is to provide a wide range of accurate and timely information on all aspects of haemophilia and related disorders. Haemophilia is a dynamic specialty and therefore opinion may change or be varied from time to time.

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THE H WORD

I hope you are all keeping safe, and preparing for the colder winter months ahead. As New Zealanders have adjusted to the new Covid-influenced normal, HFNZ is pleased to be able to offer events and workshops for our members once more. We are looking forward to Women's Weekend in Dunedin in June, and then our National Family Camp confirmed for Ngāruawāhia in April 2023. In between, we are planning for a Youth Leadership Workshop, an Adult Weekend, and regional activities. Events like these are important, because, not only do they provide an opportunity for education, they also foster the crucial connection between members that makes HFNZ such a close-knit organisation.

As we have been planning our upcoming events, many people have asked what our policies are for keeping members safe from Covid-19. At HFNZ we will always abide by the rules set by the Ministry of Health. Of course, the venues we use are able to have their own requirements over and above the MOH minimum, and we will adhere to those.

Another way that HFNZ supports members is by developing good leaders. This year HFNZ treasurer, Hemirau Waretini, is completing AFFIRM training. AFFIRM stands for Adult Fellowship for Integrating Responsible Mentors, and is an international advocacy and leadership development program. It is designed to mentor men and women with a bleeding disorder as they further develop and expand their leadership and advocacy skills with the aim to bring better care, programmes, and treatment to their respective communities.

This is a two-year commitment for Hemi, and involves a range of learning that will greatly benefit HFNZ at a Board and operational level. We congratulate Hemi on being accepted into this programme, and look forward to his ongoing contribution to our kaupapa.

It was lovely to see parts of Aotearoa lit up red for World Haemophilia Day again this year. I am reminded at this time every year just how far we've come. As an organisation, and in terms of treatment and care. HFNZ members are now able to lead long and satisfying lives with their bleeding disorder, and the increased availability of novel therapies like Hemlibra gives hope for the future. With this in mind, HFNZ are currently working to determine the needs of our members, as they grow older. Former Outreach Worker Sarah Elliott is leading this project. We look forward to her report, as we look at how we continue to support our members.

Deon York

HFNZ President



From the CEO

BY SUE ELLIS

As I noted in my December report the challenges of the Covid-19 pandemic was a preview of what was to come. Rising case numbers and, sadly, deaths from the virus have caused a pause in our day-to-day activities and a need to manage our lives with great caution.

There has been a general feeling of unease and disconnection as members, unwilling to meet in person, and uncertain about visitors and exposure, worry about close contacts or have Covid in their household. This has also affected staff morale, so our zoom meetings have become important for sharing how we're feeling and coping, and just connecting with one another.

The high number of cases has also led to low registrations for events. Feedback from members is that they are either unwilling to attend as they are unvaccinated, or they are not wanting to be exposed to contracting Covid. However, with a lessening of restrictions we may begin to see light at the end of the tunnel! Regions are now starting to see members keen to attend events.

I am sad that we have had to say auf wiedersehen to our Outreach Worker Rosie Maguire from Southern, as she departs for her big OE to teach at a kindergarten in Germany. Rosie brought a lot of inspiration and energy to her role, using her initiative to get things off the ground for Southern members. She has been a valued colleague and team member and although we will all miss her, we wish her well for this exciting journey. Hopefully we will see her again when she returns to New Zealand.

Although our plans to hold a Women's Weekend in Dunedin in November were disrupted by Covid restrictions, we are now fast approaching the rescheduled event on 10 to 12 June - a long anticipated event. I'm sure with the programme we have in place it will be an exciting time and worth the wait for the attendees. We look forward to reconnecting and experiencing a great programme.

Planning is progressing well for the long awaited National Family Camp at the Ngāruawāhia Christian Youth Camp. This was originally booked for April this year. However, with concerns about contracting the virus, registrations were very low and not enough to make it a viable event. Needing to postpone this important event, we were fortunate to have our booking rolled over until 11 to 14 April 2023. We also have a leadership development day booked in for 10 April.

The youth leadership face-to-face meeting on 26 March in Wellington was unfortunately changed to a virtual meeting due to the increasing high case numbers of Omicron being reported in Wellington city. The purpose of this meeting was more of a social get together to plan for a more formal face-to-face later in the year. Feedback from youth in the regions to ORWs was a general disappointment not to be connecting face-to-face as anticipated. A date for this to happen later in this year will be made as soon as possible to keep the interest and momentum up from our young members.



Despite all matters related to Covid, the national office has been very busy undertaking two important projects to support members. The Older People research project Te Roopu Rangatira: Our Holders of Knowledge is being led by Sarah Elliot, who some of you may remember when she was the ORW for Northern and undertook a research project as part of completing her Master's degree, exploring issues and challenges for older men with haemophilia. The purpose of this research as a follow-on from our 2020 survey, is to ensure we are meeting the needs and support required for all our members aged 55 and over. A questionnaire was sent out on 31 March and feedback is slowly coming in.

The focus of the Women's Bleeding Disorders project is two-fold. Firstly, to develop information packs and resources for primary healthcare services and teachers related to the issues and challenges for young girls and women with bleeding disorders. Secondly, to help address the burden of the high cost of sanitary products. We are approaching companies that produce period products to argue for reduced costs of their products and underwear. We are also working to see how we can work together with Period Place.

I want to thank the staff who, despite the challenges, have continued to work throughout this pandemic to ensure members are supported. They have taken the opportunity to come up with innovative ideas to reach out to members to keep the momentum going through different activities and offerings.

With the ongoing pandemic, we know this virus will be in our lives for some time to come, so I encourage members to be fully vaccinated in order to attend HFNZ events. We can continue to be strong and resilient through this ongoing uncertainty, and being fully vaccinated will ensure we are managing the risk.

As I close off, remember to wear face covering when required, and keep you and your whānau safe and well if you are travelling around New Zealand.

Period Stories

MY BLEEDING DISORDER

BY TINEKE MAOATE

This is my 46-year journey about how my period controlled my life. I was born with von Willebrands Disorder and another Platelet Disorder. So, I was on a difficult path from the beginning. I got the pleasure of beginning my change into womanhood at the age of 12, going on 13. I was fortunate to have a mother who has vWD too, so was aware of what was in store for me. Sadly, she had spent her first 20 years not knowing of her bleeding disorder, so her periods were extremely heavy and uncomfortable. However, this was normal for her, and all she knew. She tried to prepare me for what would be a huge challenge. This might sound dramatic, but I hope my story can help everyone to understand.

When my periods started, it was a couple of months of not too bad. I thought, "Hey, I've got this". Little did I know that this was just the calm before the storm. Wow, when they started to flow they really started to flow. This became the new normal for me. Being young, I only used pads. However, I didn't have much faith that they would hold up to the challenge. They didn't! I had to change pads hourly, and my pants too at times. I had to be very aware of everything I did. How I stood. If I sat down, would there be a mess when I got up. That dreaded feeling that you have leaked through your clothes. Standing with your back against the wall. Going to the bathroom a lot to check yourself out or to change another pad. Teachers not understanding that you required the bathroom often. I had to give my life story in details for them to excuse me from class. I'm sure some of them didn't believe me, but it was funny to watch the male teachers squirm when you mentioned the word period. The high blood loss was only part of it. There was also the cramps, the feeling of weakness and tiredness, and the mental drain of trying to keep on top of it. It was a dirty little secret you held onto. I was even too embarrassed to talk about this with my friends.

Mum was the one forking out for sanitary items. I'm sure she took on extra jobs just so she could afford these for myself and my sister. My mother was a widow, so finding that sort of money, on top of the other essentials, was hard. I now have a deeper understanding, being a mother of three girls. My 26 year old has lived a seemingly parallel life to mine. She too has struggled with heavy periods and all the complications that come with having a bleeding disorder. There were times when the cost of the sanitary items were so costly that she had trouble affording them when she went out on her own. Fortunately, my husband and I could always help her out. Now our two other girls, aged 11 and 12, have begun their own severe period journeys. Like their mother and their big sister, both also require medication and medical intervention to deal with their condition. That means that, once again, the cost has hit home. The high cost of such basic necessities puts young women with bleeding disorders and their whānau at an unfair disadvantage.

Period underwear has been a god-send for giving confidence to my girls. No, it doesn't lessen the flow, but it helps make the feeling of leakage less of a concern. They still usually require a pad too, but there are some odd days where they can get away with just the underwear, albeit with frequent changes. It breaks my heart to see my daughters going through it now. I believe that periods need to be discussed more openly at school and at GP visits. On many occasions, doctors have told my daughters and me that what we were going through was normal. YEAH RIGHT! What's normal?

On top of all of this there is the bleeding disorder related treatment, which many of us have to go through to help us. From tablets to infusions, and then the longer-lasting devices that we have inserted in us to help relieve the pressure. The multiple days off school or work to deal with heavy bleeding is another huge cost, with the education you are missing at school, and your pay in the real world. I have been lucky with my hysterectomy, in that I have been able to eliminate the need for a lot of treatment by removing a big part of the problem. This was after having six children though. I felt it was my last and only option, as I had tried everything else.

I feel that the government needs to be covering the cost of ALL sanitary products for ALL girls. There needs to be a routine check-up for girls to have a talk about their health and concerns. This could bring to the surface many matters, including periods, their mental state, and many other concerns. Maybe this would identify any problems, which could lead to earlier intervention.



Haemophilia testing in women and girls

Your questions answered

Genetic testing • Factor level testing



HAEMOPHILIA FOUNDATION AUSTRALIA

New HFA Education Resource

Haemophilia Foundation Australia has published a new education resource, Haemophilia testing in women and girls: Your questions answered. It's a comprehensive booklet about genetic testing and factor level testing in haemophilia for women, girls and parents of girls.

There are often many questions and things to think about when checking to see if a woman or girl is affected by haemophilia.

- What is involved in genetic and factor level tests?
- Who should have them, and at what stage in their life?
- How is haemophilia passed on in a family – and what if there is no family history?
- And why do some women and girls have bleeding symptoms or haemophilia and others do not?

HFA developed the booklet to answer these and other questions in collaboration with women and parents in the Australian community, Haemophilia Treatment Centres and genetics and legal experts. It includes infographics, tables and personal stories from community members Jane, Sharri and Michelle.

Download the booklet from the HFA website - <https://tinyurl.com/haemophilia-testing-WG>

You can also download specific sections if you are interested in a particular topic - for example, genetic testing and counselling.

If you are interested, take a look at the resource and pass it onto anyone you think would find it helpful.

Also, look out for the short and simple version, which is coming soon!

HFNZ Short Story Competition Winners

Over the summer, Northern OR Darian Smith organised a short story competition for our younger members. The stories were to be less than 500 words long, and to include the theme 'summer'. A huge thank you to all our young members who sent in stories for this over the holidays. They were all fantastic stories and fun to read. You made it difficult to pick our winners but here goes.

FIRST PRIZE (\$50 PREZZY CARD):

Josh and Steve's Summer accident!!

Josh is a person with healing powers. He can run as fast as a cheetah and jump as high as 30 meters in the air. He is the most loyal and kind person there could ever be. His best friend is called Steve. And his favourite season of the year is Summer. One of the reasons Josh loved Summer was savouring those delicious lemonade ice pops. Another was spending hours on the beach.

This particular Summer a significant event took place in the lives of these two friends. Josh was spending time at Steve's house. It was such a terribly hot day and Steve had a magnificent idea of jumping on the tramp with foam bubbles. They asked Steve's Mum if they were allowed, but Steve's Mum said no. They were confused and Steve was angry so he went against his mother, grabbed the bottle of bubble liquid and turned the hose on. Josh thought about telling Steve's Mum, but then thought this might be lots of fun so he went onto the tramp too. They were both having a refreshing blast when all of a sudden Steve screamed in pain clutching his hip and fell to the floor of the tramp. Josh quickly stopped jumping and asked, "What's happening? What can I do?" Steve exclaimed that it was probably a hip bleed and he was incredibly sore. So Josh grabbed Steve's hand to comfort him and immediately Steve somehow had no more pain in his hip. The pain had completely disappeared. Josh grabbed Steve's other hand and pulled him up.



Just at that moment, Steve's Mum came rushing to them and asked "What's wrong, are you boys ok?" The two boys said that Steve just had a hip bleed. Steve's Mum eyed the blue tramp they had and gave an "I told you so" look at Steve and then said "Do you now understand why I said no Steve?" "Yes," he answered. She looked at Josh, "If you didn't know Josh, Steve has a blood condition called haemophilia, you can search it up when you get home. Basically, he bleeds a lot. This is the reason I didn't want you having foam on the tramp as it makes it extra slippery and causes accidents to happen easier. Steve can't be too careful with his bleeding disorder."

"Yes, Mrs Rose," Josh said, "Thanks for letting me know." Then the boys went inside to watch a movie.

During the movie, Steve whispered to Josh, "Dude, how did you do that power healing thing? That was super duper incredibly amazing." "I know, right," Josh said, "Maybe I should have a superhero name." "Yeah, like Healing Man or maybe Ambulance Boy," exclaimed Steve. "Nah, what about Captain Healer, it's got a nice ring to it," Josh answered. "Yeah, I think so too," said Steve ... "CAPTAIN HEALER!"



When Josh turned 22 he did become a superhero and he did get into the saving people business. Now he saves boys and girls with haemophilia around the world. In fact just the other day Josh saved a boy who hurt his head and started bleeding. And a girl caught in a fire who started bleeding like you wouldn't imagine. His goal is to save everyone he can.

HFNZ Short Story Competition Winners

Name: Noah Gordon

Age: 12

Region: Northern

Judge's comments: I loved this story. It has superheroes, adventure, surprise, and even manages to pull in bleeding disorders. What an ideal summer it would be with this healing superhero helping people with haemophilia around the world. Brilliant.

SECOND PRIZE (\$25 PREZZY CARD):

Hi my name is Raukokore Stirling and I am 8 years old. I live in Manurewa with my mum Cassie, my dad Te Ahuriri (T.A) and my brother Rigel who is 4years old.

My school hasn't opened since the lockdown in August, so I had to stay home for 4 months and couldn't even play outside with my friends. It was a very long time to just stay home and do schoolwork and chores.

When the border opened in Auckland, Rigel and I went on an aeroplane to Gisborne with our Nanny. We could play and swim with my cousins in their pools, have sleep overs and go to the movies. There was lots of hot days so we always went to the beach, and I love swimming in the big waves and trying to ride the waves on the paddleboards. It was really good to be out with all the family and having lots of fun.

We stayed in Gisborne for 4 weeks and Papa drove us back to Auckland, it was a 6 hour ride. It is good to be home and I'm excited to go back to school. I hope Covid goes away and there are no more lockdowns

Name: Raukokore Stirling

Age: 8

Region: Northern

Judge's comments: This story captures the strange experience Kiwis have had the last few months, both the frustrating lockdowns and a delightful Kiwi summer at the beach, just like we all love! I also hope Covid goes away! I couldn't agree more.

THIRD PRIZE (BOOKMARK):

On one summer day there was a fish called Travis Bubbles his favourite thing to do was rap. He was fishy famous. That summer's day he planned to play at the lost city of Atlanta. There were 100,000 fishys at the concert but unlucky for them it was fishing season and 3,000 fishys were fished up. Then the fisherman went home and cooked them on the BBQ. Travis Bubbles decided never to do a concert in summer again.

Name: Jimmy McCabe

Age: 10

Region: Wellington

Judge's comments: This delightfully imaginative flash fiction story made me laugh. What an unexpected ending! I'll never look at rap or fish the same way again. I loved it.

Ageing with Bleeding Disorders

As bleeding disorder treatment and care gets better, people with bleeding disorders are living longer. That means that common age-related issues are affecting them more and more. HFNZ offers many services and supports to older people with bleeding disorders. But do we do enough?

This year HFNZ are looking at how we support our older members, and whether we can change or improve what we do.

Former Outreach Worker Sarah Elliott is the lead researcher for this project. Sarah is highly qualified for this due to her past work for HFNZ and her Masters' research on ageing with haemophilia. She is currently the site coordinator for the social work degree at the Eastern Institute of Technology.

In the first instance we have surveyed members aged 55 or over, who have a diagnosed bleeding disorder. The questionnaire covers a variety of topics, from how HFNZ perform, to what other supports and services members access in their communities. Completing the questionnaire will help us to plan future supports and services.

Once we have collected data via the questionnaire, Sarah will collate it and analyse it for common themes. Members who complete the questionnaire are also being offered an opportunity to be part of a focus group, or a one-on-one interview, so they can share their views personally. The information we gather from the questionnaire will help guide the direction of these meetings.

Following the individual and group meetings Sarah will combine her findings into a report, which will be used to inform decisions about the supports and services HFNZ offer to our older members.

This is an important piece of work, and we are looking forward to the information that comes from it. For more information contact us at info@haemophilia.org.nz or by calling 03 371 7477.



Events Preview

During the last two years of Covid restrictions, it's been difficult to hold many of our regular events, and we've really missed them. Even though restrictions are now easing, many of us are still understandably wary of meeting in larger groups, and of leaving the relative safety of our familiar places. With that in mind HFNZ have been working towards getting our regular schedule of national events back up and running.

At HFNZ we work hard to offer a range of events designed to cater for members of all ages and stages. From our National Family Camp, for families of newly diagnosed children, to Adult Weekend, we want to ensure that we are supporting the needs of members throughout their lives.

Coming up in the next 12 months we're looking forward to running some of our key national events.

WOMEN'S WEEKEND.

Dunedin. 10 - 12 June 2022.

Traditionally haemophilia and bleeding disorder education is geared towards men. This residential workshop specifically targets women with bleeding disorders, and women who carry the haemophilia gene, of all ages. Women's Weekend offers a safe and supportive environment where women can increase their knowledge; and discuss issues that relate to their age and stage, or to being a carrier of the haemophilia gene.

This year's event, in Dunedin, is already fully subscribed. Attendees have a fantastic programme lined up for them, including some inspiring speakers and workshops, and the opportunity to get out and about socialising.

Keep an eye out for the full report in the next issue of Bloodline.

NATIONAL FAMILY CAMP

Ngāruawāhia. 11 -14 April 2023.

Young families, where a child (0-10 years) is newly diagnosed with a bleeding disorder, often need support and information to manage the impact of the disorder on their family. The National Family Camp enables parents to access education in a supportive environment, while their children are enjoying their own recreational programmes. Opportunities are included for families to mix and mingle so that they can establish friendships and develop support networks with other families who understand and live with bleeding disorders.

This key event in the HFNZ calendar was originally planned for April 2022. Sadly, Covid restrictions forced a postponement, and now we're looking forward to April 2023. If you have a newly diagnosed child in your whānau, and you haven't attended National Family Camp before, this is the best place to start your HFNZ journey. With parent-focused education sessions, and many child-centred activities, you'll come away with a whole new understanding of your child's bleeding disorder, and a support network to match.

If you're interested in registering your interest in this event, contact us at info@haemophilia.org.nz.

Women's Weekend 2018





ADULT WEEKEND

National Family Camp 2019

Planning is in the early stages for an Adult Weekend before the end of the year. Probably in October or November, when the weather's beginning to get warmer again.

Living with a bleeding disorder, or living with a person with a bleeding disorder, is challenging. Adult Weekend is an opportunity for adults with bleeding disorders to openly discuss their experiences, challenges, and concerns about living and aging with a bleeding disorder. The focus of the weekend is to increase knowledge about bleeding disorders, about upcoming trends and treatments, about aging with a bleeding disorder, and about the provision of health care in New Zealand, as well as learning about HFNZ and its activities. Adult Weekend also includes partners, and the weekend is valued by members as a great way to reconnect.

Keep your eye on your inbox for more information about this event.

Of course, there will also be assorted regional events as the year rolls on. Like the national-level events, these regional activities provide a fantastic opportunity for members around the motu to get together, share some stories, and learn from one another.

Adult Weekend 2020



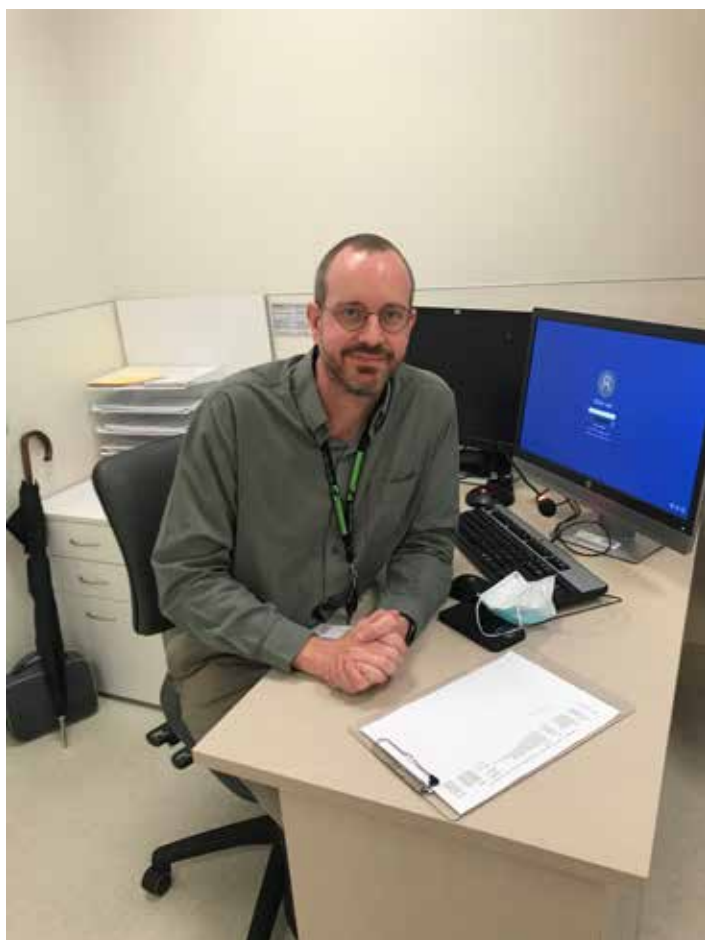
Update from the Midland Haemophilia Treatment Centre

BY MAUREEN CAMPBELL

As you may or may not be aware Dr Julie-Anne Bell has now left the haemophilia service, we thank her for her years of service.

The remaining team members are Dr Julia Phillips, and Mr Abhi TikkiSETTY – specialist haemophilia physiotherapist.

We would like to introduce the newest member of the team, Dr Gustavo Faulhaber. We are indeed lucky to find a person of his calibre to join us and serve the bleeding disorder population in the Midland Region NZ. Dr Faulhaber is a specialist haematologist. He had his training in Brazil, having a broad experience in both clinical and laboratory Haematology where he attained a Masters and PhD degree in Medicine. Dr Faulhaber's interests are in medical education and research in Haematology Pathology.



Gustavo Faulhaber

Dr Faulhaber came to NZ two years ago with his wife and 12-year-old daughter. Working at Waikato Hospital and path lab. He is very happy to be part of the haemostasis team for the Midland Region New Zealand.

Before coming to the Waikato Hospital Gustavo was Professor at the medical school of the Federal University of Rio Grande do Sul, in Brazil, and Director of Zauol Haematology lab, which is the regional reference laboratory for the diagnosis of haematological disorders. He has worked with haemostasis and thrombosis patients for more than 15 years

Other news, we are introducing a new virtual clinic for our mild bleeding disorder group. This will take the form of a yearly update via email and will ensure that no one is lost to follow up, and not losing the direct contact links with us for any problems in between follow up times

The launch date for this was Friday 22 April 2022. The first patients to be included in this clinic will have received a flyer explaining the process.

As usual, I am still here to take your calls and always look forward to hearing from you all.

Ngā mihi,

Maureen - Clinical Nurse Specialist Haemophilia for the Midland Region

Around the Branches

The first half of 2022 has been a quiet time for the HFNZ regional branches: Covid certainly has a lot to answer for. However, with restrictions slowly easing, and people becoming more comfortable with the new normal, we are beginning to return to regular programming.

In the **Northern** region members were finally able to attend the long-planned rock-climbing event. While not too many participated, those who did enjoyed the opportunity to reconnect with other members.



During this time Northern Outreach Worker Darian Smith has been able to reflect on his first few months in the HFNZ whānau:

My first 9 months with HFNZ have been enjoyable, rewarding, and challenging. I've loved meeting many of our members and clinicians and attending the fantastic Youth Camp in Christchurch. Then, of course, came the lockdowns and Covid outbreaks which have made meeting up and conducting in-person activities a lot more challenging! But thankfully we have phone, email, zoom and can still stay in touch in different ways. I was able to run a short story competition for our younger members and look forward to other creative fun.

One of the useful opportunities lockdown gave me was the chance to go through our database and update people's details. Many members will have had me contact them asking for their current emails, phone numbers and addresses etc. If you haven't, but think we might not have things up to date, please get in touch and let me know!

I look forward to meeting all of our members in person as things settle down again and am available to contact in the meantime for support, information, or just a chat.

In **Central**, they have come to realise over the last few months more so than last year is that it is the face to face contact that they crave. The free flow of side conversations and anecdotes and stories shared amongst members that Zoom just doesn't provide. They are looking forward to being able to come together as a region later in the year for their bi-annual camp. They believe that this is something positive to focus on that will provide the much needed contact local members have missed as we move back to being able to strengthen the relationships we have made over the years, and hopefully make some new ones.

The **Southern** crew enjoyed a special World Haemophilia Day outing to the International Antarctic Centre. This was very well attended, and all participants really appreciated the opportunity to reconnect after such a long break. They enjoyed a guided tour of the facilities including experiencing an "Antarctic Blizzard", the penguin display & then a shared cuppa & scone.



Sadly, Southern Outreach Worker Rosie Maguire recently moved on. She has taken the opportunity to experience life in another part of the world, taking up an early childhood teacher position in Germany. Rosie has been excellent for the Southern region, and will be missed.

Things have remained very quiet in the **Midland** region. However, while there have not been any events, there has certainly been activity in the region. Haematologist Julie-Anne Bell has moved away from her role in the Waikato Haemophilia Treatment Centre, after many years excellent service. HFNZ members appreciate the time Julie-Anne put into her role at the HTC, and thank her for all her hard work. Julie-Anne has been replaced by Dr Gustavo Faulhaber.

As things settle down on the Covid front we look forward to more opportunities for regional events.

BBM-H901, Gene Therapy for Hem B, Shows Efficacy at 1 Year in Pilot Trial

BY STEVE BRYSON

The gene therapy BBM-H901 increased factor IX activity in 10 men with moderate to severe hemophilia B, while reducing bleeds and the use of FIX replacement therapy over one year, according to results of a pilot Phase 1 trial.

“The study was the first clinical trial conducted by Belief BioMed in China,” Jane Zheng, PhD, CEO of Belief BioMed, the therapy’s developer, said in a press release. “We are very proud to introduce BBM-H901, which is the first intravenously delivered gene therapy drug for hemophilia B in China and the first example of systemic gene therapy for rare diseases in China.”

Findings were reported in the study, “Safety and activity of an engineered, liver-tropic adeno-associated virus vector expressing a hyperactive Padua factor IX administered with prophylactic glucocorticoids in patients with haemophilia B: a single-centre, single-arm, phase 1, pilot trial,” published in *The Lancet Haematology*.

In hemophilia B, mutations in the F9 gene lead to a lack or production of a defective version of the blood clotting protein factor IX (FIX), resulting in uncontrolled and prolonged bleeds. Standard treatment is replacement therapy, in which the missing FIX protein is infused into patients to avoid or stop bleeds.

BBM-H901 aims to restore FIX production by delivering a functioning copy of F9 packaged in a modified, harmless adeno-associated virus (AAV) to liver cells, where blood clotting factors are made.

The open-label Phase 1 trial (NCT04135300) evaluated the safety and activity of a single intravenous (into-the-vein) infusion of BBM-H901 in 10 adult men in China.

Eligible participants had a pre-treatment FIX activity of less than 2 international units per deciliter of blood (IU/dL), no antibodies against the FIX protein, and low levels of antibodies targeting the AAV. Patients received regular doses of the immunosuppressant prednisone for one week, followed by a single dose of BBM-H901 at 5×10^{12} vector genomes per kilogram of weight (vg/kg).

Participants were monitored for treatment-related side effects, liver toxicity, and the development of antibodies against AAV. FIX activity, bleeding rates, and replacement therapy use were also evaluated.

Within the first week of infusion, mean FIX activity reached 57.1 IU/dL, peaking at 64.1 IU/dL at a median of five weeks. The mean increase in FIX activity over the median follow-up period of 58 weeks (just over one year) was 36.9 IU/dL.

The median annualized bleeding rate dropped from 12 to zero, the median number of target joints decreased from 1.5 to zero, while the median number of FIX replacement therapy infusions fell from 53.5 to zero. Of note, target joints are joints where bleeds occur frequently.

There were no reports of serious or severe adverse events. One patient had a fever and another elevated levels of a liver enzyme, a sign of liver toxicity. Although no anti-FIX antibodies were seen, all developed antibodies against the AAV after the infusion.

“This pilot study suggests that liver-tropic BBM-H901 is safe [one] year after infusion,” the researchers wrote. “Vector derived FIX:C concentration is sufficiently high to prevent bleeding events and minimize the need for replacement therapy in small populations with hemophilia B.”

Its findings “support further study,” they added.

After the one-year of follow-up, participants were given the option to enroll in an extension study evaluating the safety of BBM-H901 for up to five years.

“The BBM-H901 study is the first clinical study in China and even Asia to utilize liver-targeted AAV vectors for the treatment of hemophilia B,” said Zhang Lei, MD, a professor at the Chinese Academy of Medical Sciences.

“The safety and long-term effectiveness of this treatment strategy have been well demonstrated with significant relief of associated complications,” added Lei, who is also vice director of the Institute of Hematology and Blood Diseases Hospital at the Chinese Academy.

Source: <https://hemophilianewstoday.com/2022/05/27/bbm-h901-hem-b-gene-therapy-shows-efficacy-safety-pilot-trial/>

CSL-UniQure Hemophilia B Gene Therapy Scores Priority Review

BY HANNAH CHUDLEIGH

Biotechnology leader CSL Behring announced its Biologics License Application (BLA) for etranacogene dezaparvovec, an investigational treatment for hemophilia B, has been accepted by the U.S. Food and Drug Administration for priority review. If approved, it would be the first gene therapy treatment for hemophilia B.

Hemophilia is a degenerative genetic disease in which the gene that produces clotting Factor IX (FIX) in the blood does not work properly. The result is that the patient does not have enough FIX for their blood to clot properly in case of blood vessel damage, and patients can experience bleeds in their muscles and internal organs. This can lead to painful, swollen joints and, in some cases, death.

To treat hemophilia, CSL Behring and its partner, uniQure, use an adeno-associated virus (AAV) to make more normal clotting possible in the patients. In AAV, a modified, non-infectious virus enters target cells with “instructions” for the cells to start producing proper levels of FIX. After delivering the instructions, the virus dissolves.

Pennsylvania-based CSL Behring submitted the BLA for the genetic treatment after gathering positive data from its HOPE-B trial, a pivotal, open-label, single-arm study that tested the safety and efficacy of etranacogene dezaparvovec. The 54 participants in the trial first completed a six-month measuring period in which they established a baseline Annual Bleeding Rate (ABR).

After that, patients received a single dose of etranacogene dezaparvovec. The primary endpoint in the pivotal HOPE-B study was to measure the participants’ 52-week ABR after achieving stable FIX expression, as compared to their original six-month established baseline ABR.

The results of the study showed that a single infusion of the biologic reduced the adjusted annualized bleeding rate by 64%. The rate of FIX-treated bleeds was reduced by 77%. By the end of the 52-week period, 98% of subjects treated with a full dose of etranacogene dezaparvovec discontinued use of the prophylaxis FIX treatment.

“The acceptance of etranacogene dezaparvovec for review by the FDA brings us closer to our goal of delivering a life-changing treatment option for people with hemophilia B,” said Bill Mezzanotte, M.D., MPH, executive vice president, head of R&D and chief medical officer at CSL Behring. “Etranacogene dezaparvovec, potentially the first gene therapy approved for hemophilia B, further demonstrates CSL’s promise to relentlessly pursue innovative and disruptive technologies when it benefits rare and serious disease patients with unmet medical needs.”

Previously, the European Medicines Agency (EMA) accepted the Marketing Authorization Application (MAA) for etranacogene dezaparvovec under its accelerated assessment procedure. Now that the biologic’s BLA has been accepted, the FDA will review it within 6 months, rather than the standard 10-month timeline.

The news is also a huge milestone for uniQure, which has led the multi-year clinical development program for etranacogene dezaparvovec. The company develops single-treatment gene therapies for genetic diseases.

Source: <https://www.biospace.com/article/fda-accepts-bla-for-potential-first-hemophilia-b-gene-therapy-treatment/>

Haemophilia support: AR Joint Scanner unveiled at the CHW

An Australian-designed, augmented reality (AR) technology unveiled at The Children's Hospital at Westmead (CHW) will give young people living with haemophilia a glimpse into the future and help them stay on track with treatment.

Haemophilia, an incurable, inherited rare blood disorder, is estimated to impact more than 3000 Australians.¹ It is diagnosed when there is not enough clotting factor VIII(8) or IX(9) in the blood to help control bleeding in the body.¹ This bleeding most commonly occurs in the joints of the knees, elbows and ankles,² and can lead to joint disease if not treated adequately. As haemophilia is also an inherited condition diagnosed at birth, joint disease caused by haemophilia can begin to develop as early as the age of 20.^{2,3}

The AR Joint Scanner, which will be used by clinical staff at the Kids' Factor Zone at Westmead Children's Hospital's Paediatric Haematology Unit, will help educate young patients and their families on the possible future impact of joint disease and the importance of maintaining a regular treatment program to help prevent bleeding episodes.

The scanner uses a 'leap motion' 3D camera attached to a computer to scan and map a person's hand when placed under the device. A specially designed software then overlays imagery onto the user's hand to replicate normal aging and the impact of joint disease.

While the scanner uses only the user's hand as its reference point, it cleverly allows the user to expand the replicated view on the screen beyond the hand to see what is happening elsewhere in the body, focusing on specific joints known to be impacted by haemophilia including the shoulder, knee or ankle.

"Technological advances like augmented reality, seen in this Joint Scanner, allow healthcare professionals to bring important health education to life that directly engages their patients. We know improved health education can help patients make informed decisions about managing their health conditions," said Murray White, Joint Scanner developer from Crash Bang Wallop.

Tim Demos, a 28-year-old Melbourne-based ambulance worker who lives with haemophilia A, said the Scanner provides an insightful and startling lens into what life can look like if he doesn't continue to pay attention to his haemophilia management.

"I've always felt I had a pretty good handle on managing my haemophilia, but even so, it's easy to disregard the impact of my condition in the next 10 years. This Scanner has helped to strengthen my understanding that what I do now will determine how I live with my haemophilia for the rest of my life", Tim said.

Sanofi has developed the Augmented Reality Joint Scanner, which is provided on loan to hospitals across Australia, including Westmead Children's Hospital, as an educational resource for patients and their families.

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Source: <https://www.hospitalhealth.com.au/content/clinical-services/news/haemophilia-support-ar-joint-scanner-unveiled-at-the-chw-1125557250>

Project GYM: Physical Fitness Improves Wellbeing in Young Men With Hemophilia

BY CALEB RANS, PHARMD

A hemophilia-specific fitness training program, termed Project GYM, was shown to improve psychological wellbeing and self-confidence in young men with hemophilia aged 18 to 25 years. ^{1,2}

“Many young men with hemophilia engage in physical activity and sport, but face challenges to participation because of their [condition],” wrote Paul McLaughlin, MSc, of the Katharine Dormandy Haemophilia Centre and Thrombosis Unit at the Royal Free London NHS Foundation Trust in London, UK, and his coauthors.¹

The researchers published the results of 2 studies on the safety and feasibility of Project GYM in *Research and Practice in Thrombosis and Haemostasis*.^{1,2}

Nonblinded, Randomized Feasibility Study

A nonblinded, randomized study was conducted to evaluate the feasibility of Project GYM, and its effect on gym activity, motivation, and adherence to exercise. The researchers included 19 young men with hemophilia A or B (all severities, with or without inhibitor) from 3 hemophilia centers in London.

All study participants were provided an activity tracker and gym membership, and were randomized to “gym only” or “gym and personal trainer” groups. Questionnaires were administered at baseline and 6-months to evaluate participants’ motivation to exercise, physical activity levels, quality of life, self-efficacy, and self-esteem.

After analysis, the researchers found that participants within the “gym and personal trainer” group had greater gym attendance compared with the “gym-only” group. Specifically, 7 participants increased their activity levels, while 9 remained the same, with no statistical difference between the groups.

The median Hemophilia Joint Health Scores (HJHS) improved in 3 participants, but were unchanged in 12. Moreover, there was no bleeding related to gym activity.

Observational Feasibility Study

Kate Khair MSc, PhD, of the same institution as Mr McLaughlin, led a separate observational study in the same cohort as the randomized study. She and her team used thematic analysis to analyze individual audio-recorded interviews about study participation.

After analysis, they found that there was a significant difference in motivation to exercise as shown by the Stages of Change grouping moving from contemplation to action and maintenance phases ($P = .03$). In addition, self-efficacy overall scores trended towards improvement, but were not statistically significant ($P < .06$).

Furthermore, the median self-esteem scores of participants improved from 22 (range 12-30) to 25 (range 13-30), and were significant ($P = .02$).

“Key themes identified from the interviews were: fear, self-confidence, “being normal,” pain, weight loss, ability, and getting fitter,” Dr. Khair and coauthors wrote.

“The psychological wellbeing of young men with hemophilia improved during this study, [which] may have been related to participating in a gym-based, physical exercise program,” they added.

Future Directions

Importantly, this was the first study to evaluate a nonmedical gym environment and personal trainer-led physical fitness program in people with hemophilia. Overall, the results from both studies showed that training with personal trainers led to greater gym attendance and participants felt more supported compared with “gym-only” intervention.

“[We] highlight the need to [better] understand support needs in future studies,” the researchers concluded. “Behavior change theory and techniques should be included when investigating gym-based activity for young men with hemophilia.”

“In the future, we would love to replicate this study, particularly in women, and we are currently seeking additional funding to continue the project,” Dr. Khair said in an email interview.

Disclosure: Some guideline authors have declared affiliations with or received funding from the pharmaceutical industry. Please refer to the original study for a full list of disclosures.

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Source: <https://www.hematologyadvisor.com/home/topics/bleeding-disorders/hemophilia-project-gym-physical-fitness-improves-young-men-patients-risk/>



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