Bloodline

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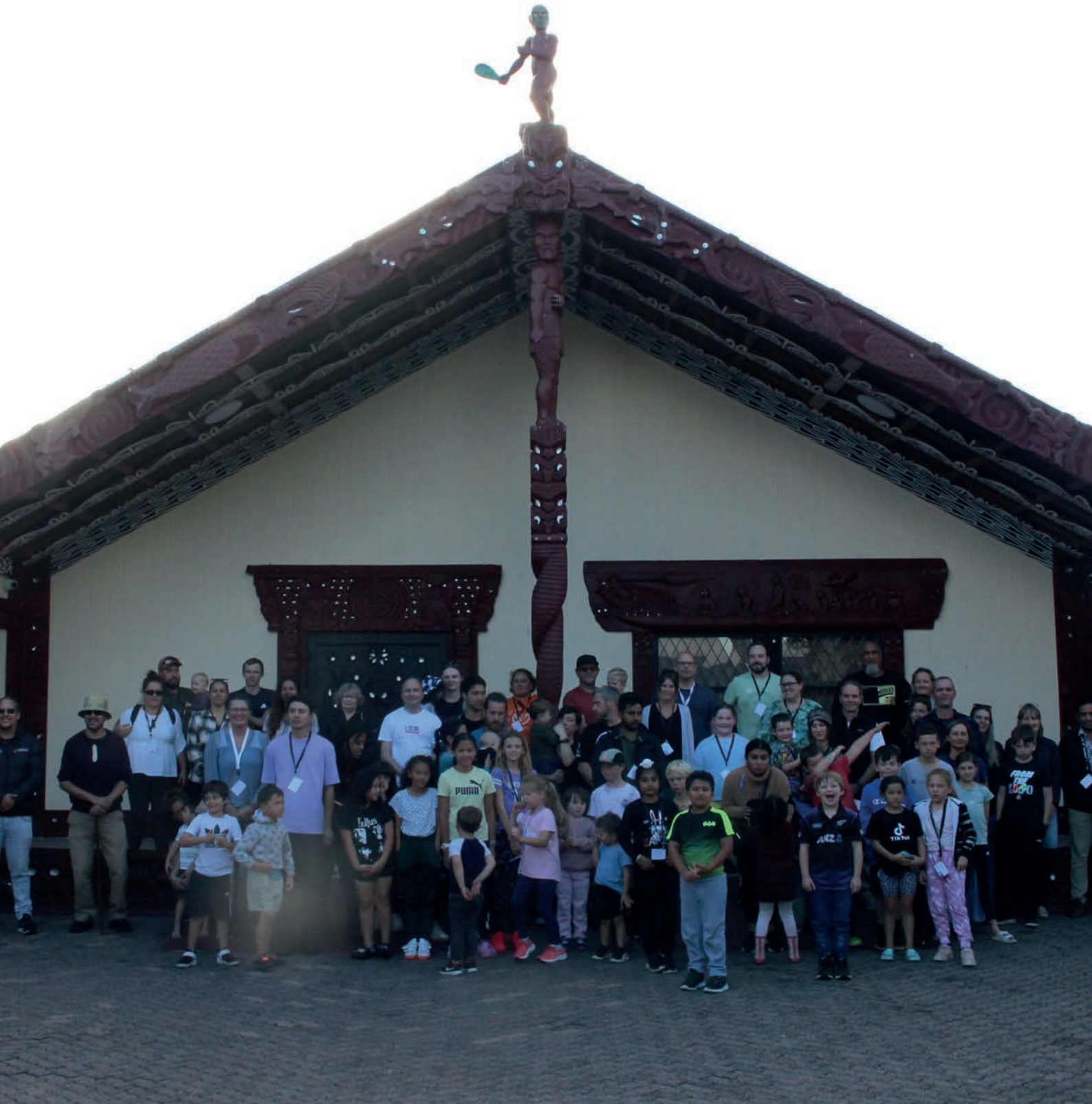
Haemophilia Foundation of New Zealand Inc.
HFNZ



Bloodline

Magazine of the Haemophilia Foundation of New Zealand. Volume 51 Number 1

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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

It has been a while, but your latest edition of Bloodline is finally here. It has been a very eventful time for HFNZ this year as we continue to increase the opportunities for members to connect once more and emerge from the years that have been.

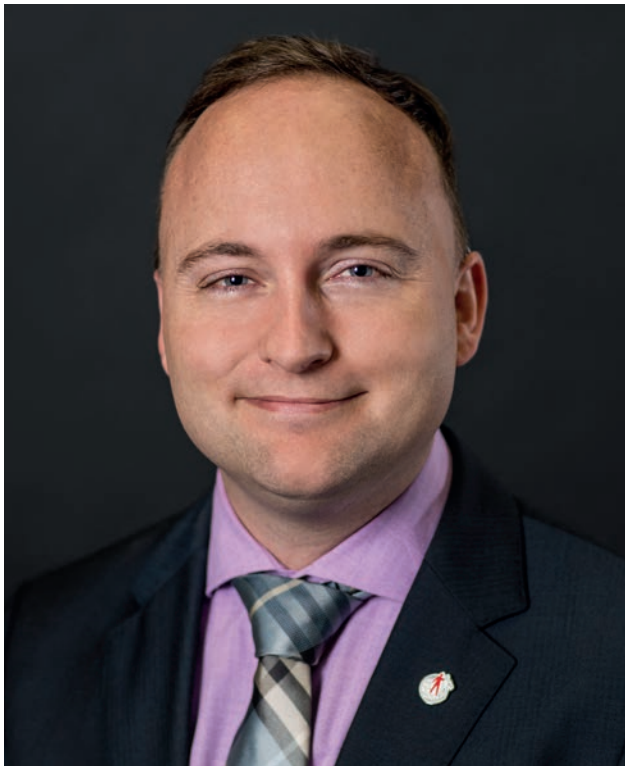
I am excited to announce that Vic Turner joins HFNZ as our Southern outreach worker. Vic brings a wealth of experience with her, and we are delighted to have her on board to become part of the HFNZ team and the community. Vic, Southern has been waiting and I am sure simply can't wait to meet you! Thank you to Darian, Loren, and Lynne for covering the South Island as we searched for the right person to look after the Southern region.

In April we were delighted to finally run our national family camp in Ngāruawāhia. Some participants had only recently received a diagnosis in the family, so learning about the fundamental clinical aspects of bleeding disorders was particularly important at this event alongside connecting with other families. Sessions for parents covered 'haemophilia 101', dental care, information about novel therapies, including Hemlibra, and progression towards gene therapy. A session on the importance of physiotherapy was also run and a 'parents panel'. One of the participants self-infused his treatment for the first time, and parents learnt how to self-infuse on 'willing veins'. Thank you to staff for pulling together and making this camp such a success. Many volunteers also made this possible, but I must single out someone who doesn't like a fuss: The camp could not have happened without Tineke (also a World Hemophilia Day star in the Women's Weekly!). Thank you, Tineke, on behalf of all of us! Another 'shout out' must go to Tuatahi and Te Whainoa who arranged an unexpected and uplifting visit to Tūrangawaewae marae. I would also like to thank our sponsors Roche, Rātā Foundation, Pub Charities, and Waikato Trust for supporting this event.

HFNZ is affiliated with Rare Disorders New Zealand (RDNZ) and they have had an active year to date. On 28 February (Rare Disease Day), I attended the launch of 'Raising a child with a rare disorder: A guide for parents living in Aotearoa New Zealand' at Government House. The event was hosted by RDNZ's patron Her Excellency, the Right Honourable Dame Cindy Kiro and was attended by many people living with a rare disorder, proving the adage of RDNZ that 'rare is everywhere'.

On May 10, I also attended the launch of RDNZ's BERL Rare Disorders Insights Report at Parliament. This report is based on publicly available data and a high-level literature scan of recent New Zealand and international studies that discuss the prevalence of rare disorders, as well as burden factors that need to be considered in the development of health policies. Both resources are available on RDNZ's website, raredisorders.org.nz

We continue to await the decision from Pharmac on the funding of Hemlibra. We know that this therapy is game-changing for people with haemophilia A. We hear from members about the equity gap continuing to grow as we have a group of members who receive this therapy and a



group who do not. We continue to advocate on your behalf. We also have an eye on the reforming health system and any unintended consequences for our members.

For those following the property development, you will know that HFNZ purchased a property and took possession at the end of 2022. This property has one tenant, and room for our national office. We are working through a basic design to modify the existing space and have it office ready. Stay tuned for an official opening! The timeframe will depend a lot on builder availability. Read into that what you will. We are also undergoing an overhaul of our branding and will be able to share our revamped logo and brand identity in the coming months once the finishing touches are completed. The concepts were developed with a focus group of members from a range of ages and stages.

Finally, there are several events coming up in the coming months (and years!). Mark them in your diary now as we would love to see you there!

- Youth weekend 15-17 September 2023 (Wellington)
- Youth sailing event – January 2024 (Auckland)
- Teen and youth camp – 1-3 March 2024 (Auckland)
- Women's wellness weekend - 26-28 July 2024 (Taupō)
- Family camp - 6-9 February 2025 (Ngāruawāhia)
- Adult wellness weekend - Oct/Nov 2025 (Christchurch)

For more information on any of the events listed above, please contact info@haemophilia.org.nz or your local outreach worker.

Ngā mihi,

Deon York

Chair and Acting CEO

Haemophilia New Zealand

2023 National Family Camp: Ngāruawāhia

On 11 April nearly 90 people from around the motu gathered in Ngāruawāhia for the 2023 National Family Camp (NFC). This number included 17 whānau, along with leaders, staff, and clinicians. We were also pleased to welcome members of the HFNZ board to this very special event in the HFNZ calendar.

Young families, where a child is newly diagnosed with a bleeding disorder, often need support and information to manage the impact of the disorder on their family.

The NFC 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.

Usually, the age range for children with bleeding disorders at camp is 0-10. For this camp, the age limit for children was raised to 13 or under. This reflects the impact of Covid on our events and workshops, and that we've been unable to run a National Family Camp since 2019.

Members rolled in through the afternoon of Friday 11 April. It was lovely to see so many smiling faces after such a long wait for this event. Before the camp started, the weather forecast had been a concern. This was borne out on the first evening, when the heavens opened during our first activity: marshmallow toasting on the campfire. Luckily, there was some shelter for s'mores eating, and we headed back to the main hall for introductions and dryness.

Day two saw parents head off to the first of their education sessions, while our fabulous youth leaders entertained the children. Special mention must go to Louise Tupou, who ran our crèche. This was a tough job, which Louise and her team did very well.

The education sessions on day two started with the traditional parents' panel, where a group of parents was able to share their BD journeys, and respond to members' questions. This was followed by a presentation from the Waikato hospital dental team, before haemophilia nurse specialist BJ Ramsay talked about the basics of haemophilia and von Willebrands. In the afternoon, Julia Phillips talked about the latest advances in BD treatment.

While all this was happening, the older children built and sailed their own rafts before going kayaking, and the younger group spent time creating animal masks.

Later in the day, we were very privileged to be welcomed onto the Tūrangawaewae marae. This very important marae is the home of Tūheitia Paki, the Māori King. It was very moving to see the historic buildings and taonga, and hear some of the history associated with the marae and the Ngāruawāhia area. This visit really was a highlight.

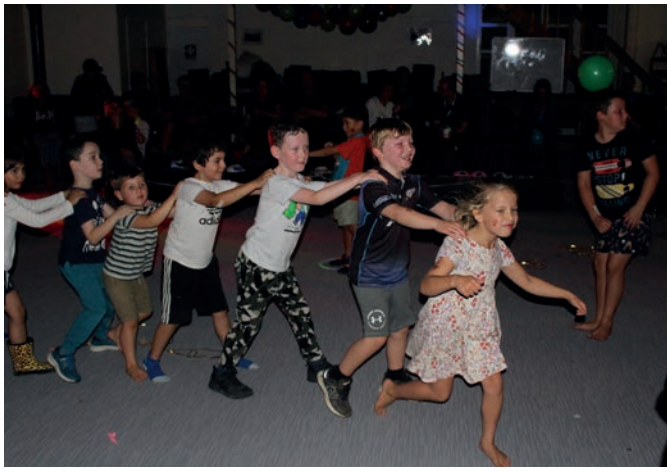
The big HFNZ disco rounded out the day. It was awesome to see kids and parents up dancing to the beats. I'm sure there were some tired bodies afterwards. The guest DJ did a great job, and the kids thoroughly enjoyed the games and fun.

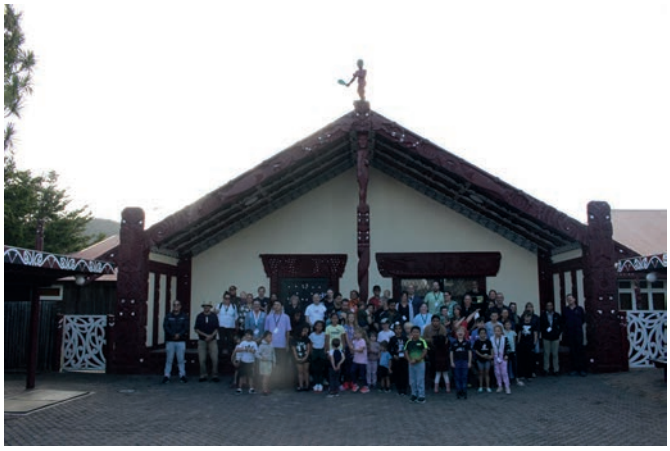
Day three started with Deon talking to the parents about the history and future of HFNZ, while the kids learned about the clotting cascade with BJ. Activities and education continued throughout the morning, before the whole group headed off to Hamilton Zoo for the afternoon. This was a fantastic trip, which was enjoyed by all.

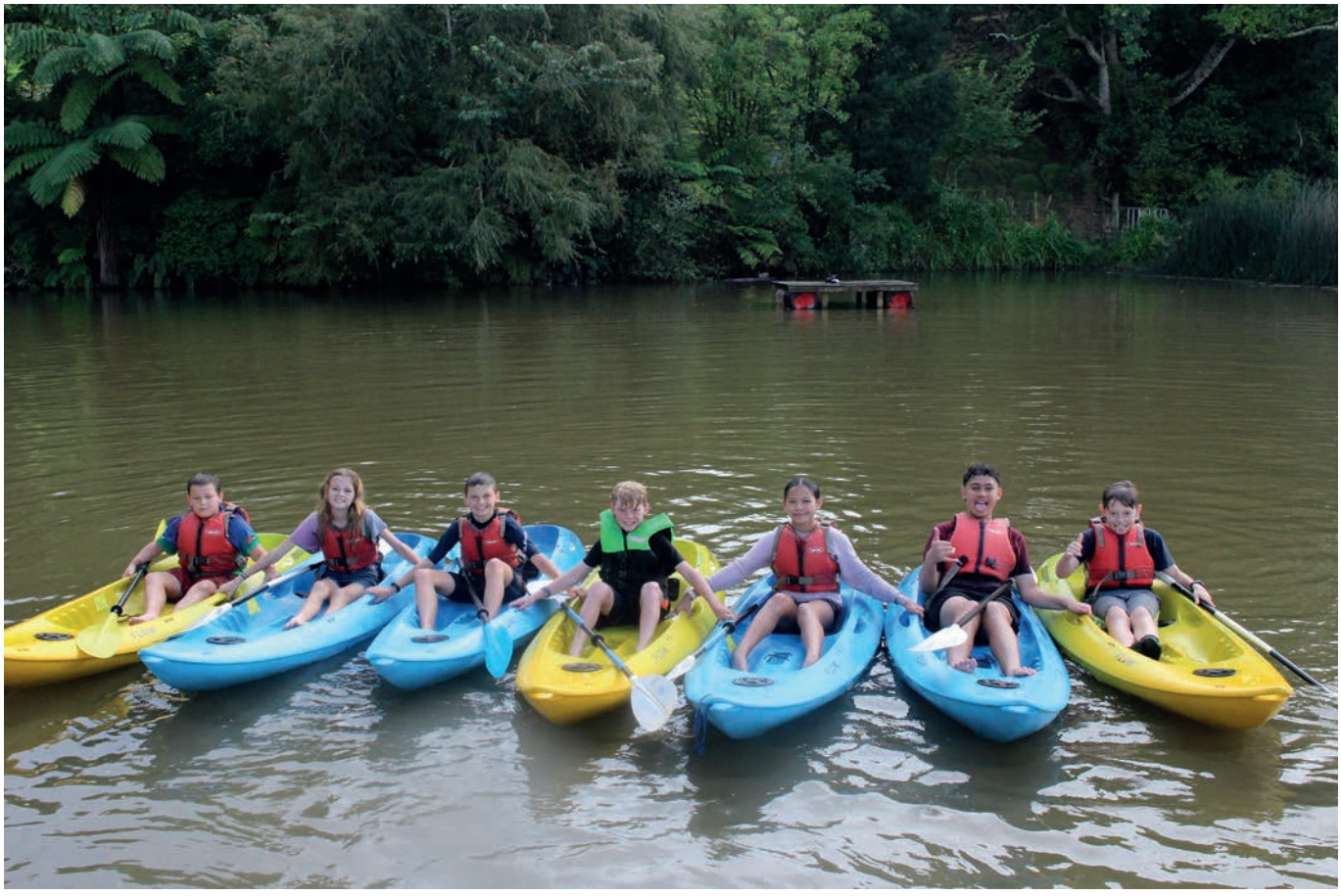
After a couple of full days, the final night was movie night for the younger members, while their parents gathered to share and socialise. This was the perfect way to finish a very successful camp, with tired people all around enjoying the chance just to relax.

This event would not have been possible without the support of a wonderful group of leaders. A big thank you must go to Louise, Jessamyn, Evekanya, and Eshka for all their hard work in crèche, along with Benedict, Gabriel, Anna, Nicholas, Brendan, and Victoria for supporting the other children through a range of activities. The energy and enthusiasm this group added to the camp was priceless.

Finally, we'd like to thank Roche, Rātā Foundation, Pub Charities, and Waikato Trust for contributing funding towards this event. Without their support, we would not be able to offer such high quality events and activities to our members.







The Marvellous Ms. Maoate

By Hayley McLarin

Republished with permission from the New Zealand Woman's Weekly

As a teenager, Tineke Maoate's daughter Teagan begged her mother for a hysterectomy.

Living with a genetic blood disorder, she desperately wanted to stop the excessive bleeding during her period and was willing to never have a child of her own to be freed from the ordeal.

Tineke, 47, knew only too well what she was going through, as she also suffers from multiple bleeding conditions which causes extreme blood loss each month, prolonged bleeding if injured and while giving birth to her six children.

"I did take Teagan to see a specialist – but he said he ethically couldn't do it when she was so young," says the King Country woman, who chose to have a hysterectomy herself at 35 after her youngest child was born.

"I wanted it earlier but I didn't meet the criteria even though I had von Willebrands Disorder and another platelet condition."

In hindsight, both Tineke and Teagan, now 26, are pleased the procedure wasn't done, with the arrival of grandson Sylas five months ago. Yet Tineke is sure much of the trauma and angst all of her children, who were diagnosed as babies, go through could be avoided if there was greater awareness of their condition.

For the females in the family, it can mean periods so heavy that they need to replace sanitary products hourly, while her sons have faced bullying by fellow pupils, who know they won't respond as they cannot risk being injured.

"The bleeding it controls you – the women in our family have to think ahead. Where's the toilet going to be? And you bleed for weeks – affecting things like intimacy. That takes a very understanding partner," says Tineke who spoke to The Weekly ahead of World Haemophilia Day on April 17.

Her husband of 24 years also has a blood disorder, resulting in a unique mutation for their children, twins R-Leeo and Theodore, 23, Tarquin, 19, Evekanya, 13, and Eshkar, 12.

"It hasn't controlled me but it's had a different effect on my children. They have disabilities and we cannot take the risk of contact sport. I home school the younger ones too, so they aren't bullied about it," shares Tineke.

Her twin sons were born at 27 weeks and also have asthma and lung conditions. Theo is autistic and deaf and Tarquin has ADHD.

"ADHD with a bleeding disorder is not good. He wants to climb and jump off things," says Tineke.

"He got really badly bullied but would never fight back because he worried they would hit him in the head and a head injury for a person with a bleeding disorder is really serious. That can cause a bleed on the brain way worse than a normal person."

Home schooling, raising her family and keeping them safe is a demanding and constant job but the busy mum takes it all her in stride.

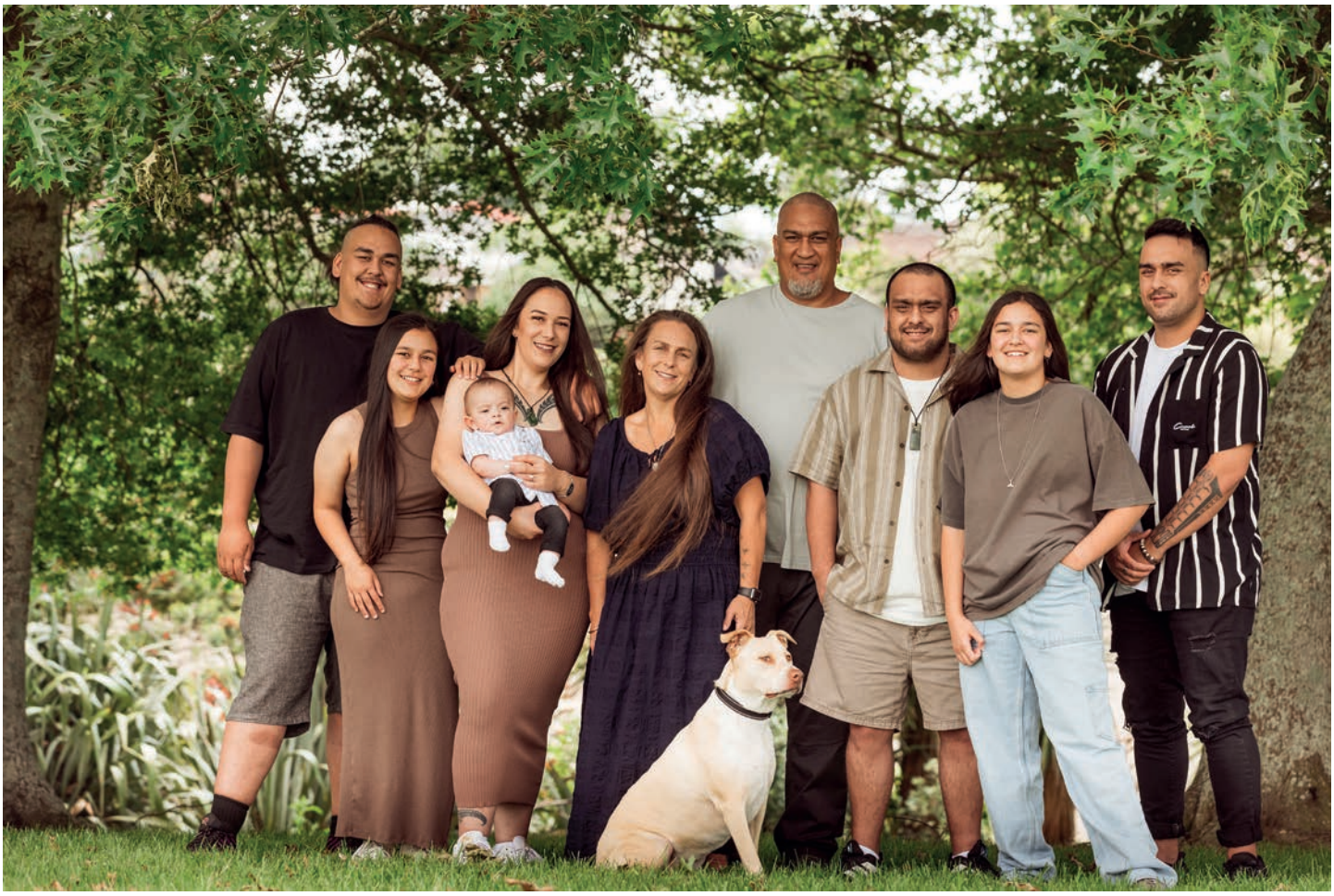
Their family also regularly takes in foster children, is a volunteer firefighter for her rural community and has run local food banks.

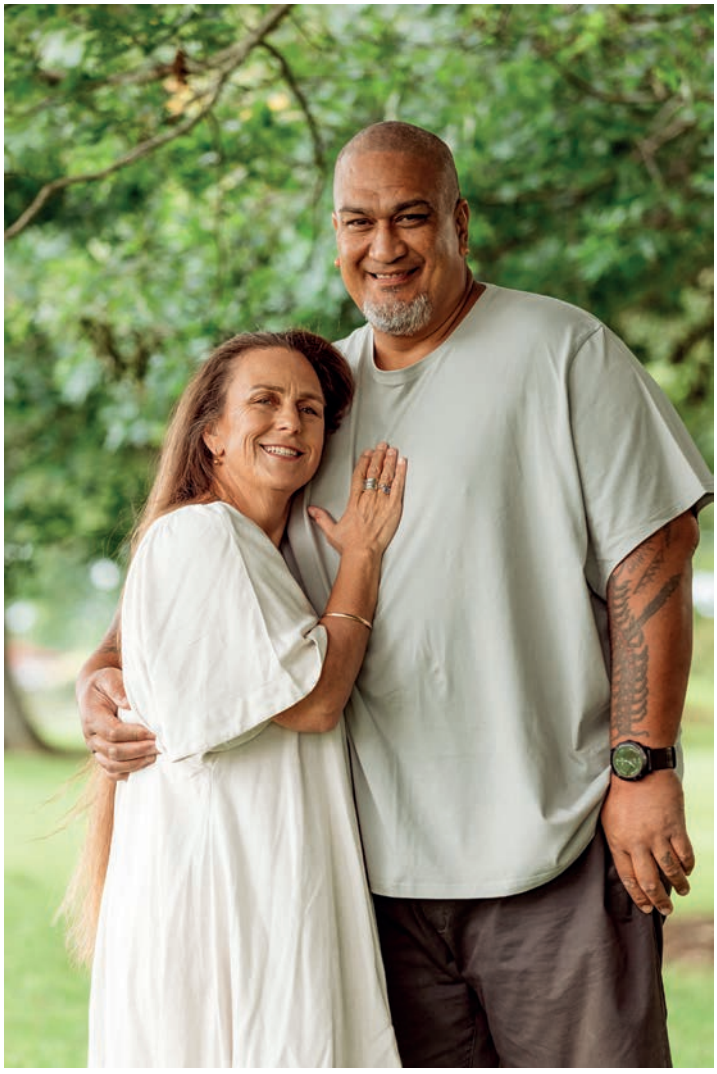
Community and giving back are so important to Tineke she even finds time to be on the Haemophilia Foundation of New Zealand board, is a senior mentor on camps and helps support others with a blood disorder.

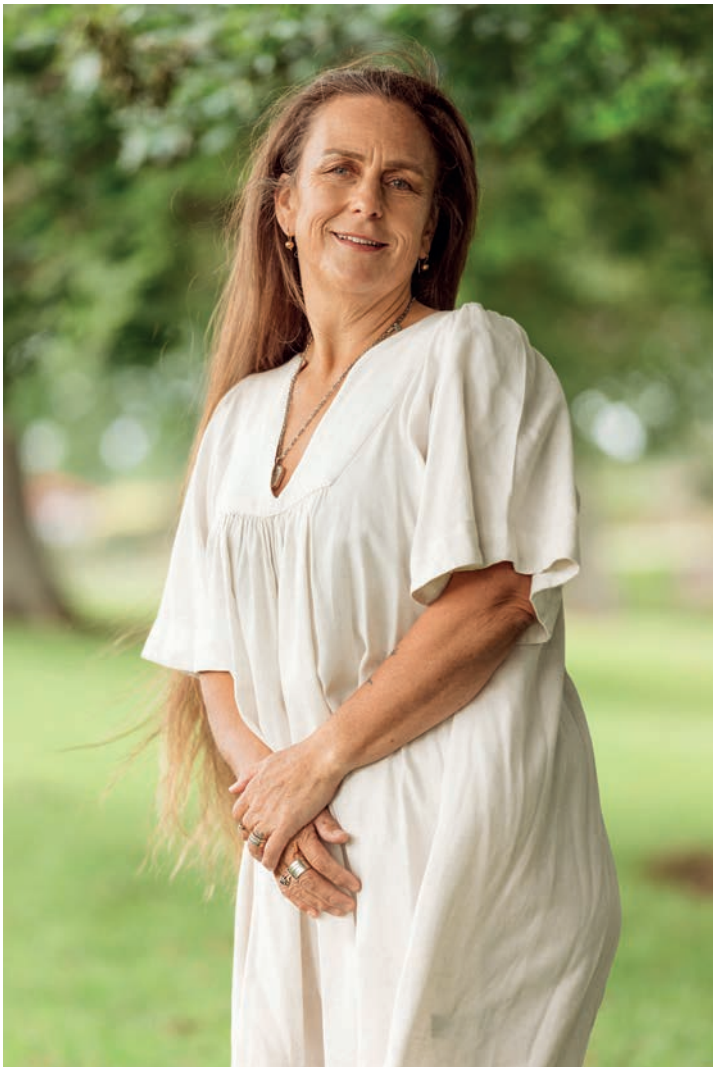
"HFNZ is my biggest passion because it affects my family so much. And we are able to provide a Pacific voice as well. We are trying to get Pasifika people to talk about it, and get support because sometimes they get diagnosed but they hide it," says Tineke. "You can live really good lives as long as it's managed and you are well looked after and supported." Tineke hopes her tireless efforts will have an impact, especially on improving care for young women.

"I wish doctors would listen when a young girl says she has heavy bleeding. A lot of our women do not find a bleeding disorder until they give birth, so there's a trauma. It can rule your life trying to work out where the toilets are and worrying will you bleed through your clothes? That's hard for a teenager."

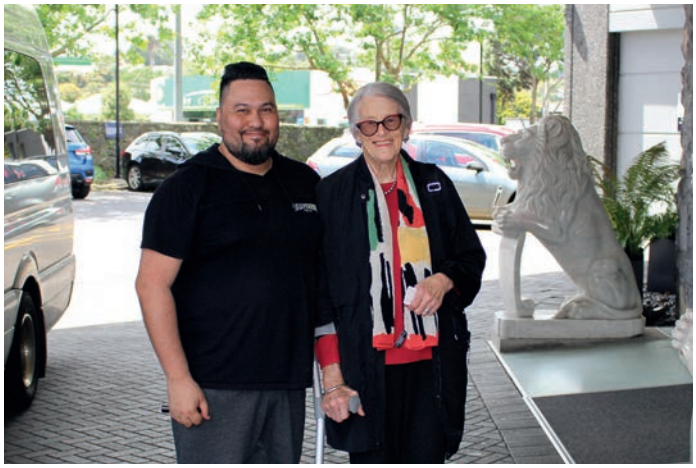
The determined mother will never stop advocating for her children and others and says it's normal to feel overwhelmed by these serious conditions. "It's okay to let them have a pity party and say 'poor me'. It's hard but you cannot let it rule or ruin your life."



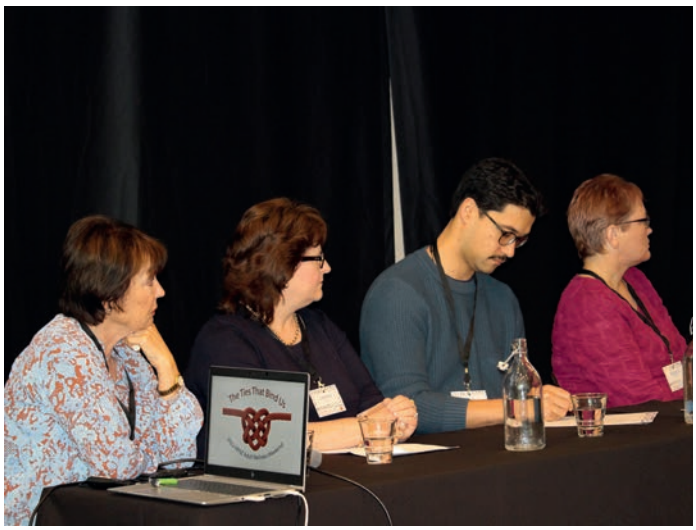




HFNZ Adult Wellness Weekend 2022



Living with a bleeding disorder, or living with a person with a bleeding disorder, is challenging. The HFNZ Adult Wellness Weekend is an opportunity for adult members with bleeding disorders and their partners to openly discuss experiences, challenges, and concerns about living and aging with a bleeding disorder.



The focus of the weekend is twofold: education about bleeding disorders, upcoming trends, and treatments, as well as socialising and reconnecting with peers from across the motu.

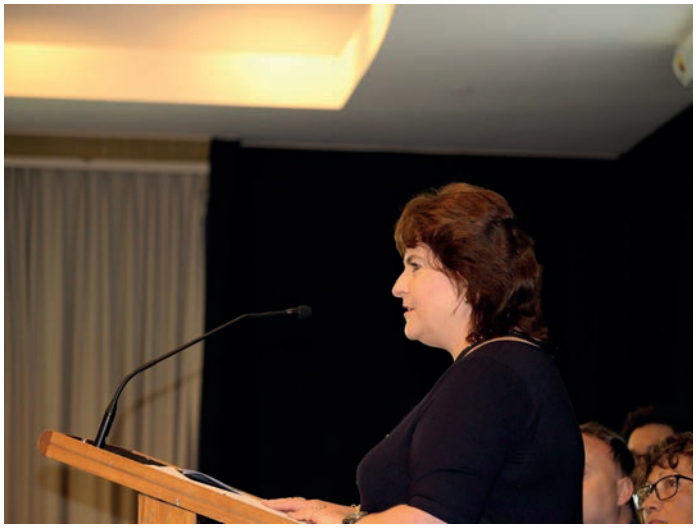
At the end of 2022, members gathered in Auckland, at the Novotel Auckland Ellerslie, for a weekend of learning and laughing. The weekend ran from 25 - 27 November. It was lovely to see members enjoying seeing one another again as they arrived from around the motu for the initial meet and greet on Friday afternoon. This highlighted one of the most important aspects of these national events: connection.

After some socialising and a bit of dinner, people gathered for the formal welcome, some knot tying, and a few words from Board Chair Deon York. The theme for this Adult Wellness Weekend was 'The ties that bind us'. Deon spoke about how important it is that we carry our history with us, and that it informs our future decision-making.

The programme-proper commenced on Saturday morning, with the traditional members' panel. Deon joined members Hemi Waretini, Kathryn MacGregor, Leanne Spencer, and Patience Stirling to share their bleeding disorder journeys. This is always a very powerful session, with the members sharing very personal and sometimes painful experiences, as well as the joys that can come with adversity. The key point that came from this session was how important it is for patients to be a part of their own medical journey, and for clinicians to listen to their patients.

Appropriately, a clinicians' panel followed the members' panel. Nurse specialists BJ Ramsay, Maureen Campbell, and Vanessa Minor, Haematologist Nicky Eaddy, and physiotherapists Cat Pollard, and Abhi Tikkisetty kindly gave us their time to introduce themselves to members and answer any questions from the floor. Coming right after the members' panel meant that the clinicians had an opportunity to respond to some of the historic issues raised, as well as answer some current questions. We very much appreciated their time and expertise.

For the next session, we split into two groups. Outreach workers Darian and Loren took one session looking at 'Strengthening the Ties'. While CEO Sue and outreach worker Tara took the other, which focused on 'Ties from the Past to the Future'. Both sessions went very well. The first group talked about love languages, and how we support and empower one-another through our words and actions. The second group discussed how what HFNZ have done in the past connects to what we do now, and how that might look in the future. It was fantastic to see members contributing and enjoying each other's company in a learning environment.



After a morning and early afternoon full of listening and learning, the rest of the day was given over to off-site activities. Again, members were able to select the outing in which they participated. One group went to the Stardome Observatory, while the other headed out on the water, catching the ferry across to Devonport. The observatory visit was quite spectacular, with awesome views of the cosmos, alongside some learning about what's what in NZ's night sky.

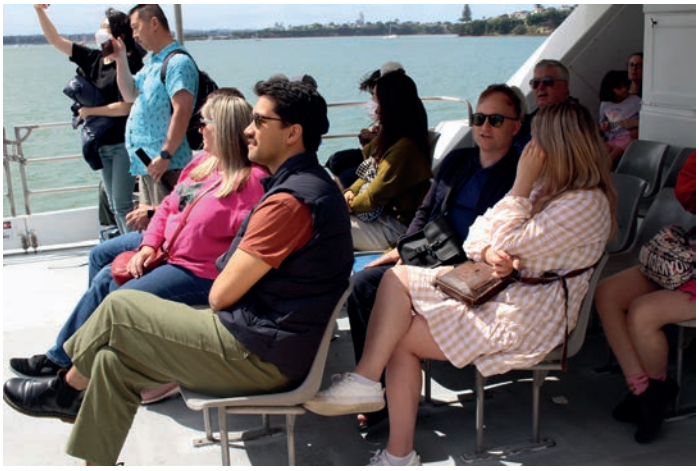
The ferry trip also went very well. After a lovely calm ride across the harbour, members spent some time wandering around Devonport village and stopping for a refreshment, before heading back on the ferry again. All had a wonderful time.

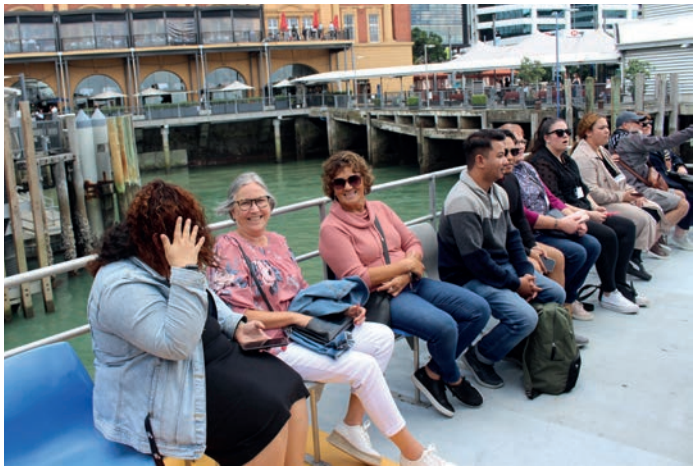
A long Saturday wound up with the, always contentious, quiz night. This edition was no exception. Teams battled it out over four rounds for the awesome solid plastic trophies, and the title of AWW quiz champs 2022. There was some intense competition, and laughter galore, before the winners were crowned, and everyone retired after a long day.

On the final day, members enjoyed HFNZ president Deon York talking about how we can all strengthen our ties with HFNZ.

This included attending events, volunteering to support member activities, joining your local committee, and feeding back to the board and staff. This was a fabulous way to round out Adult Wellness Weekend, and allowed us all to reconsider the theme of the weekend: The ties that bind us.

This very successful event was appreciated by all who attended. It wouldn't have been possible without the support of Kiwifirst, and the hard work of staff and volunteers. We're looking forward to the next one already.





Upcoming HFNZ events

The Haemophilia Foundation of New Zealand (HFNZ) supports a diverse range of members from a variety of backgrounds. One of the ways that we do this is by offering educational and psychosocial opportunities specifically targeted at particular demographic groups. Central to this are our national events. These events are generally age-targeted, and are designed to ensure that HFNZ members have the tools available to navigate each life stage.

Over the last few weeks, the HFNZ team have been planning when and where our key national events are going to be. This ensures that we can lock-in venues and book transport well in advance. It also means that members who are interested in attending these events are able to plan effectively, including taking time off work if necessary.

Here's what we have lined up:

Youth (18 - 30) event.

15 - 17 September 2023. Wellington

Youth (18 - 30) sailing.

January/February 2024. Auckland

These are open events for 18 - 30 year-old HFNZ members. The events focus on empowering young people with bleeding disorders to develop the skills and knowledge necessary to support physical activity, career paths, mentoring, and leadership skills.

Teen and youth camp.

01 - 03 March 2024. MERC, Auckland

Growing up is exciting, challenging, and often very confusing. When a bleeding disorder is added to the mix, the challenges of young adulthood only increase. The youth camp, for 11-18 year-olds, uses a combination of education, fun, and peer and mentor support, to help young people move to the next stage of managing their condition. The focus is on being well informed and better able to make decisions about their bleeding disorder treatment, education, work, life-style, and relationships.

Women's wellness weekend.

26 - 28 July 2024. Taupō.

Traditionally haemophilia and bleeding disorder education has been geared towards men. This weekend-long 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 & stage, or to being a carrier of the haemophilia gene.

National family camp.

06 - 09 February 2025. Ngāruawāhia.

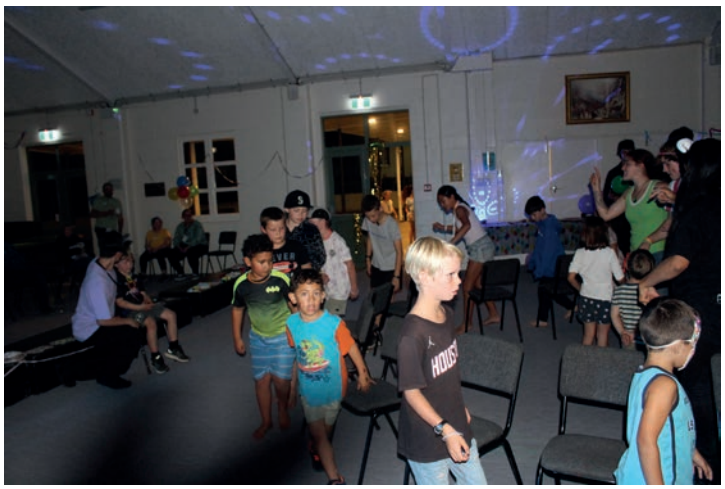
Young families, where a child (0-10 years) is newly diagnosed with a bleeding disorder, often need support and information to manage the impact of the disorder on their family. The national family camp enables parents to access education in a supportive environment, while their children are enjoying 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.

Adult wellness weekend.

October/November 2025. Christchurch.

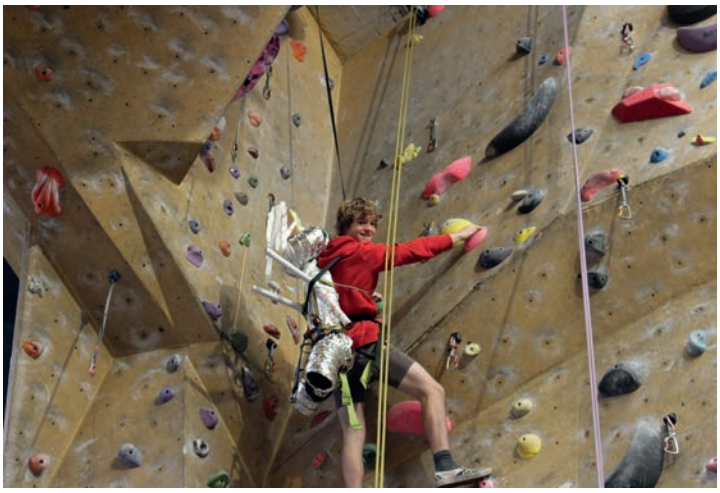
Living with a bleeding disorder, or living with a person with a bleeding disorder, is challenging. The Adult Weekend is an opportunity for adults with bleeding disorders, and their partners, to discuss their experiences, challenges, and concerns about living and aging with a bleeding disorder. The focus 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.

The goal is to run each of these national events every 2 - 3 years. However, unforeseen events like earthquakes, cyclones, and pandemics can put a spanner in the works. The best way to find out about upcoming events is to keep an eye on Pānui, or look out for invites to events targeted at you.

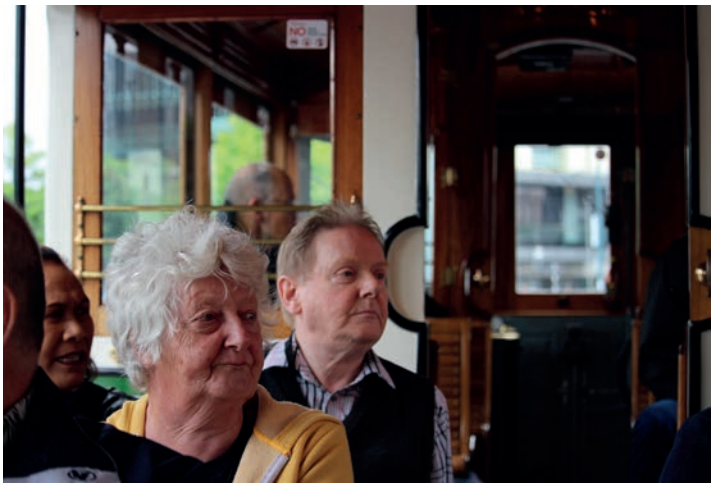


National Family Camp 2023

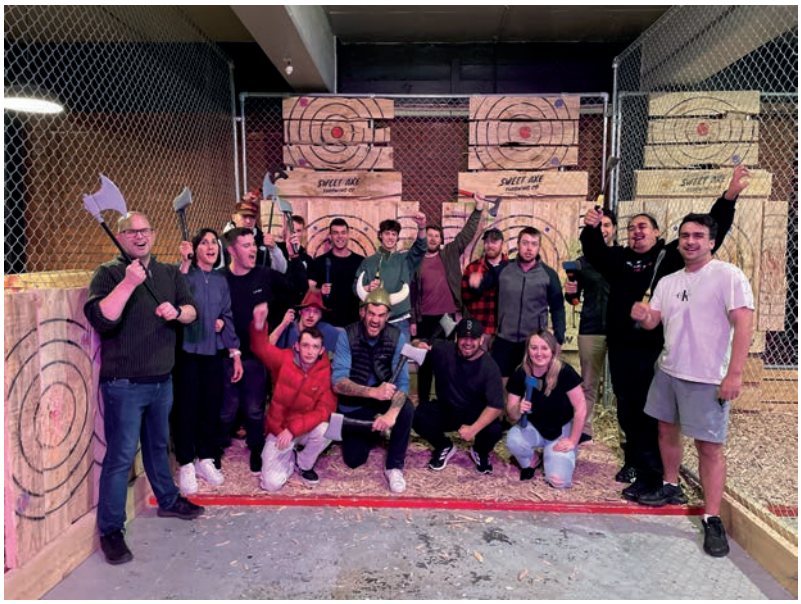
Womens Weekend 2022



Teen and Youth Camp 2021



Adult Weekend 2020



Youth Hui 2022

Welcome to Vic

After several months HFNZ are very pleased to welcome Vic Turner into the HFNZ whānau as Southern outreach worker. Vic started in her new role on Tuesday 06 June, and has quickly started getting to grips with it.

Vic comes to us by way of nursing and, more recently, counselling. Early in her career she worked at the former Templeton Hospital. Later she joined the team at Te Poutama Arahi Rangitahi. During this time she developed a strong interest in helping youth. She is particularly interested in equine assisted and adventure-based therapy, making use of the horses she has on her rural Banks Peninsula property.

Here's what Vic has to say:

"Kia ora everyone. My name is Vicki Turner although I do prefer Vic. I'm a proud 50 something wife and mother. My husband Burt and I manage a blended family with six combined offspring ranging from 35 years down to 12 years, with two Moko.

Burt, Hugo (12), and I live on our 52-hectare horse/gorse block in Okaruru bay (Goughs Bay) Banks Peninsula, Canterbury.

I originally trained as a nurse in the 80s at Burwood Hospital, then transferred to Templeton hospital until its closure. After this, I took employment at a treatment facility for young men before formalizing my training as a counsellor at Vision College. I have since specialised in equine assisted psychotherapy, which I have been using in my private practice for the last three years.

My husband and I have recently swapped positions. He now looks after the house, the horses and teaches Hugo while I have the office and begin my exciting career with the Haemophilia Foundation."

We're very pleased to have someone with such broad and relevant experience join the team. Vic has really hit the ground running, so, if you're in the Southern region you can expect to hear from her soon.

If you want to connect with Vic you can email her at vic@haemophilia.org.nz or by messaging or calling 021 656 804.

Did you know about HFNZ grants?

We all know that sometimes it's difficult to make ends meet. Even more so when you have chronic health needs. In the bleeding disorder community, some people are more affected than others are, and some people are more able to support and advocate for themselves than others. That's why HFNZ offers grants to support members accessing specific products and services.

These include:

- Swimming/Exercise
- Supportive Footwear
- Defensive Driving

Access to HFNZ's swimming/exercise and footwear grants are determined based on clinical need, and must be supported by a clinical diagnosis of a bleeding disorder that would normally predispose the member to joint and muscle problems. Bleeding disorders that automatically meet these criteria are:

- Severe factor VIII or IX deficiency
- Moderate Factor VIII or IX deficiency
- von Willebrand Disorder type 3

The swimming/exercise and supportive footwear grants may also be available to members with other related bleeding disorders. However, in these instances, a clinician, physiotherapist, or haemophilia nurse must support these applications by writing a letter or email stating that there is a clinical need related to the member's bleeding disorder. It is important to note that these grants have a cap. The value of footwear vouchers are determined by the age of the recipient, with a current maximum of $120 for adults. Swimming/exercise grants are capped at $150 per term.

Transport is essential to whānau who support a person with a bleeding disorder. HFNZ's defensive driving grant is available to all HFNZ members with a bleeding disorder regardless of clinical severity, and to the primary care-givers of children with a bleeding disorder regardless of clinical severity of the child's bleeding disorder. This grant enables members to attend a defensive driving course in their local area, up to the value of $150. These courses teach new drivers valuable skills, and reduce the amount of time between restricted and full licencing.

Sometimes other important issues arise. Perhaps you need something specific to support a person with a bleeding disorder, or maybe your budget just can't stretch to fuel for that specialist appointment. That's why HFNZ have needs grants. These non-targeted one-off grants are to help support members when they really need it. There is no set limit on the value of needs grants, or on number of times you can apply; it just needs to be related to your bleeding disorder. Each application will be assessed on its merits.

All you need to do to access any of these grants is reach out to your outreach worker. They'll let you know what you're eligible for, and help you access any other support you might need. If this sounds like something that you might be interested in, here's how you can contact your local outreach worker:

Darian Smith, Northern - darian@haemophilia.org.nz or 027 512 1114

Loren Silva, Midland - loren@haemophilia.org.nz or 021 762 121

Lynne Campbell, Central - lynne@haemophilia.org.nz or 027 273 3443

Vic Turner, Southern - vic@haemophilia.org.nz or 021 656 804

Around the branches

The Northern team had a super turnout for their combined Hobbiton event in conjunction with the Midland crew. It was great to see these two regions working together for the benefit of the members.

Northern have planned ongoing coffee group meetings at Coffee Culture in Sylvia Park. The first of these was held on 25 May, then again on 25 June. Keep your eye out for future meetings. They're also working on a visit to Butterfly Creek on Sun 23 July. The coffee group invite is out now. More Butterfly Creek details and an email invite to be distributed closer to the time.

The Central region held an awesome Christmas event last year. This was a fun time for all, and we have the photos to prove it.

The team also had a high tea on 28 May in Feilding. This was another great chance for Central members to reconnect, and doubled as a late celebration of World Haemophilia Day.

After a long recruitment process, we have now made an appointment to the vacant Southern Outreach role. As you will have seen earlier in this issue, we're pleased to welcome Vic Turner into the HFNZ whānau. There will be a welcome dinner before long, so keep your eye on your inbox. You can also expect to hear from Vic personally in the coming days and weeks.

The national youth committee are planning an event in Wellington on the weekend of 15th - 17th September. More details will be available soon.

Plans are also afoot for a youth sailing event in Jan/Feb 2024. You may remember that this was floated (haha) for late last year. Well, Hemi and the team have collaborated with the New Zealand Sailing Trust, and are looking to hold this event in the summer of 2023/24.

There is a bit happening in this space, so, if you are aged 18-30, keep your eye on your inbox!

EU Approves Hemlibra for Moderate Hemophilia Aa CDHB perspective

By Steve Bryson, PhD

The decision follows last year's recommendation by CHMP

The European Commission has approved Hemlibra (emicizumab) as a routine preventive treatment for people with moderate hemophilia A without inhibitors.

"We welcome the European Commission's decision to approve Hemlibra also for people with moderate hemophilia A in the EU," said Levi Garraway, MD, PhD, Roche's chief medical officer and head of global product development, in a press release. "As its benefit expands to broader populations, we remain committed to determining how Hemlibra might help even more people with hemophilia A to live a bleed-free life."

The approval follows last year's recommendation from the European Medicines Agency's Committee for Medicinal Products for Human Use (CHMP).

In hemophilia A, a protein essential for blood clotting called factor VIII (FVIII) is missing or defective. A lack of FVIII can lead to prolonged and excessive bleeding episodes, particularly in the joints and muscles, causing pain, joint damage, reduced mobility, and a diminished quality of life.

Approved in more than 110 countries, Hemlibra mimics the action of FVIII and prevents or reduces the frequency of bleeding episodes in people with or without inhibitors, which are neutralizing antibodies generated against FVIII that can reduce the effectiveness of treatment.

The therapy had been only approved in the EU for patients with severe disease — those having less than 1% of residual FVIII activity. People with moderate hemophilia retain between 1-5% FVIII activity, but about 85% of them still have recurrent bleeds and one-third have long-term joint problems.

The approval of Hemlibra's label expansion was supported by data from the Phase 3 HAVEN 6 study (NCT04158648) along with real-world evidence.

Even "moderate disease can produce bleeds that cause irreversible joint damage and impact quality of life," Garraway said.

The first analysis of the trial was based on data from 72 patients without inhibitors (three women, 69 men), most of whom had moderate hemophilia A (70.8%). Eligible participants reported more than 10 bleeds a year.

All the patients received 3 mg/kg of Hemlibra, given as an under-the-skin injection, once a week for four weeks, followed by either 1.5 mg/kg every week, 3 mg/kg every two weeks, or 6 mg/kg every four weeks. Patients were followed for just over a year (a median of 55.6 weeks).

Data showed 66.7% of Hemlibra-treated patients had no bleeding episodes that required treatment, while 81.9% had no spontaneous bleeds requiring treatment, and 88.9% had no joint bleeds requiring treatment.

No new safety concerns were reported in patients with moderate hemophilia A without inhibitors. Injection site reactions were the most common side effect, reported in 16.7% of participants.

About the Author

Steve Bryson, PhD Steve holds a PhD in biochemistry from the Faculty of Medicine at the University of Toronto, Canada. As a medical scientist for 18 years, he worked in both academia and industry, where his research focused on the discovery of new vaccines and medicines to treat inflammatory disorders and infectious diseases. Steve is a published author in multiple peer-reviewed scientific journals and a patented inventor.

Source: https://hemophilianewstoday.com/news/eu-approves-hemlibra-moderate-hemophilia-a/

Gen Z Speaks: I believe a bleed-free future can be a reality for people like me who live with haemophilia

By Benjamin Tang

"Each time the phone rings, my heart will skip a beat," I recall my mother saying when I was young.

When I was about six months old, I developed an unusually large bruise on my chest. My parents took me to a community traditional Chinese medicine practitioner who advised them to immediately take me to the KK Women's and Children's Hospital (KKH).

After several tests at the hospital, I was diagnosed with haemophilia A, an inherited bleeding disorder.

Those living with haemophilia lack clotting factors to form blood clots, which means that without timely medical attention, it may be life-threatening.

This diagnosis has been life-changing, not just for me, but for my whole family.

Worried about not having enough money to pay for medication, my father sold the family car.

He also changed his job from an electrical technician to a taxi driver so as to have the flexibility and a mode of transport to rush me to the hospital whenever I needed medical attention.

To supplement the family's income, my homemaker mother took to distributing flyers occasionally.

Every three to five days, my parents would have to take me to an outpatient clinic for infusion. Each visit could take between two to four hours.

When an injury happened outside of office hours, I would have to be rushed to the emergency ward where there might not be a specialist who would be able to set up the infusion procedure.

I recalled once being admitted at 7pm and only leaving the emergency department at 6am the following day.

My parents were apprehensive when expecting their second child, and only decided to go ahead with the pregnancy as tests indicated a negligible chance that this child will also have the same inherited bleeding disorder.

Growing up with haemophilia

Living with haemophilia meant being familiar with unexpected bleeding that can occur randomly.

Within an hour, the swelling around the joints can be so painful that I would no longer be able to walk.

In both primary and secondary school, my class was assigned a classroom on the ground floor so I did not have to navigate flights of stairs when I came to school on crutches or in a wheelchair, which was quite often.

My early years were sheltered. My mother did not leave me out of sight, and the flooring of the flat was padded with soft mats to cushion any fall.

At most, we would take short walks in the neighbourhood and return straight home.

I was a sporty young child with lots of pent-up energy but cautious teachers did not allow me to participate in any physical or sporting activities during my primary school years.

My only outlet then was when my parents would take me to the neighbourhood basketball court where I would be allowed to dribble and shoot a few hoops.

Other than physical activities, I was also not included in other school events, sometimes without due consultation. I grew up feeling discriminated against, left out and alone.

Despite my parents' consent, I was the only one from the class excluded from the National Day Parade (NDP) celebrations, as my primary school teachers thought there were too many steps to climb at the Marina Bay floating platform.

I recall how I was not offered the opportunity to take part in an overseas business trip while I was in polytechnic, and I did not know the reasons for it. I gave feedback to the school authorities, but to date I still have no idea why I was excluded.

Contributing to the community

In spite of focusing on the limitations due to my condition, I was determined to explore how I can be a contributing member of society.

During my time at a polytechnic, I would volunteer as a befriender to children who are sick or from disadvantaged families.

Fortunately, I had a second chance to volunteer for the NDP, which is my way to contribute to this quintessential event celebrating Singapore.

Perhaps more importantly, it was able to reconcile my feelings of being discriminated against nearly a decade ago, after my school left me out of the celebrations.

These opportunities gave me the chance to step out of my sheltered cocoon, and I relish connecting with people from different walks of life.

The best years of my life were serving in the National Service. My supervisors were very supportive and gave me opportunities to take on leadership roles.

Living with haemophilia has inspired me to be a healthcare professional to explore better and alternative treatments so that others living with health conditions can have a better quality of life and thrive.

I studied pharmacy science and have served nearly two years as a pharmacy technician at a restructured hospital.

My treatment journey

There are two approaches to treating haemophilia. Replacement therapy is used prophylactically (taken at regular junctures) to prevent dangerous bleeds, and importantly, preserve the health and function of joints.

Together with appropriate training and the use of protective equipment, people living with haemophilia can lead physically active lives and participate in a number of sports even at the competitive level.

The more affordable alternative is to seek treatment as and when needed.

Due to cost considerations, my parents had to take the difficult decision to decide against using prophylactic treatment.

So long as there is some bleeding, I will have to be sent to the hospital to receive treatment. It would require three to five treatments to truly end the bleeding. The expenses can be much higher if it is a major injury.

During my NS enlistment, I had access to medical coverage which supported the costs of treatment. My doctor advised me to leverage the coverage to switch to a newer treatment which is currently not on the list of subsidised drugs.

In those three years, there were zero bleeding episodes, which empowered me to embrace life fully.

But now that I have completed my NS, I no longer have access to the comprehensive medical scheme.

I have to give up on that treatment and revert to my former treatment regime which is on the list of subsidised drugs. Eight months later, a major bleeding episode occurred.

I am currently using the subsidised medication. Coupled with partial charitable funding from the hospital and the Haemophilia Society of Singapore, I still have to put aside half my salary for treatment each month.

Excessive bleeding in the joints and muscles can lead to longer-term consequences. Now, at 25, I have already developed arthritis, which is excruciatingly painful with each flare-up.

It is hard to imagine a future like this, but I have to make the best of my condition because this is what I was born with.

The reality is that people living with haemophilia can lead fulfilling and meaningful lives, and contribute like anybody else towards the community.

We need access to treatment for this rare disease so that it is not dependent on one's ability to pay but based on one's medical needs.

Those around us can lend support by seeking to understand the disease and be inclusive.

Educators could set an example of inclusivity. Instead of restricting students with haemophilia from regular activities, they could include less impactful sports such as swimming or frisbee and focus on involving students living with chronic health conditions.

By penning my thoughts here, I wish to invite the community to unleash the untapped potential of those of us living with haemophilia, by jointly envisaging and achieving a bleed-free future for all.

Those after me do not need to live with chronic joint pain and longer-term consequences such as arthritis.

About the author:

Benjamin Tang, 25, is a pharmacy technician at a public hospital. He is diagnosed with haemophilia A, an inherited bleeding disorder in which the body lacks clotting factors to form blood clots.

Source: https://www.todayonline.com/gen-z-speaks/gen-z-speaks-i-believe-bleed-free-future-can-be-reality-people-me-who-live-haemophilia-2190046

Gene Therapies for Hemophilia: Promising But There's Room for Improvement, Say Reviewers

By Tony Hagen

How long the gene therapies for hemophilia will keep factor levels high is unclear, said the authors of a review in the Annual Review of Medicine. Patients also need to be counseled that they can receive only one adeno-associated viral (AAV) vector in their lifetime.

The current crop of hemophilia A and B gene therapies approved or under development represents a genuinely new paradigm in care for this patient population, but these therapies are also quintessentially first-generation, and investigators see room for improvement.

"Despite repeated proof-of-concept success in current hemophilia gene therapy, stable, durable, [factor VIII (FVIII) or factor IX (FIX)] expression able to ameliorate bleeding in all patients is an unrealized hope," Benjamin J. Samelson-Jones, M.D., Ph.D., and Lindsey A. George, M.D., wrote a review article in Annual Review of Medicine published earlier this year.

Samelson-Jones is an attending physician in the Division of Hematology at Children's Hospital of Philadelphia and George is a hemophilia expert and directs the Clinical In Vivo Gene Therapy program at Children's Hospital of Philadelphia

Hemgenix (etranacogene dezaparvovec), which employs an adeno-associated viral (AAV) vector to deliver a transgene to the liver to promote production of FIX, was approved in November 2022 for hemophilia B. It reduced the yearly bleeding rate by 65%, and 96% of patients no longer needed FIX replacement therapy two years after infusion.

However, the durability of the gene therapies designed to restore FVIII and FIX levels is unclear, and there is evidence that factor expression drops over time, Samelson-Jones and George wrote in their review.

Such has been the case with Roctavian (valoctocogene roxaparvovec), a trial drug under FDA review for hemophilia A, according to Samelson-Jones and George. Early trials demonstrated "continuously decreased FVIII levels out to six years" in participants, as well as a 40% loss of FVIII expression from year 1 to 2 in a phase 3 trial cohort, they wrote.

Gene therapy holds out the potential for lifetime normalization of FVIII and FIX levels following a one-time infusion, but many patients with hemophilia A and hemophilia B may not be eligible. "To date, only adult men with endogenous factor levels ≤ 2% and without advanced liver disease have received gene therapy," noted Samelson-Jones and George.

Of particular concern is the role of AAV-neutralizing antibodies (NAbs) in modulating the effectiveness of gene therapy for hemophilia. The presence of a high degree of NAbs is correlated with gene therapy failure, and so prospective clinical trial participants with NAbs have been screened out. However, assays for NAbs are not fully reliable, and better tests are under development.

Anti-AAV NAbs can be acquired through natural infection, and environmental exposure accounts for an approximate 30% NAb seroprevalence.

Another potential problem is that NAb prevalence builds up significantly following gene therapy administration, which means that in the event of therapeutic failure, patients will never be eligible for another AAV-based gene therapy.

"The current lack of a proven strategy for vector re-administration is among the most salient considerations for clinicians and hemophilia patients when deciding on a gene therapy product," wrote Samelson-Jones and George.

"Patients should be counseled so that they are fully aware that the current state of clinical development only permits one lifetime systemic AAV vector administration," they said.

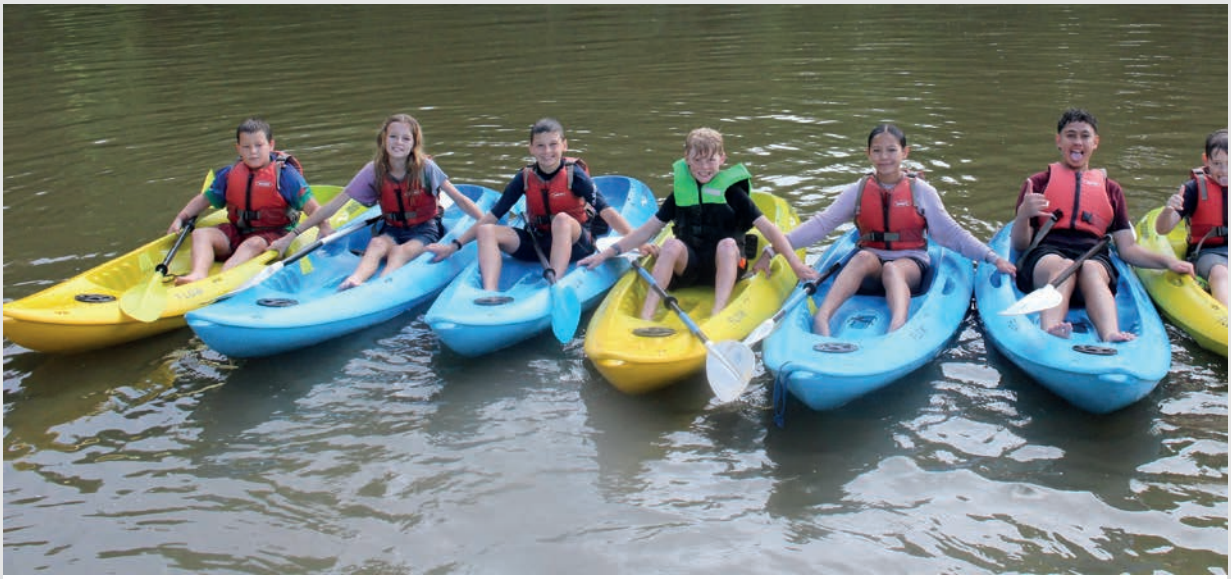
For payers, the prospect of a multimillion-dollar tab for a failed gene therapy, which would then be followed by additional costs for a lifetime's return to standard-of-care, could be sobering. In hemophilia A and hemophilia B, the standard of care is infusion of FVIII and FIX concentrate up to several times a week to achieve hemostasis.

After being accepted for clinical trial participation and being administered gene therapy, some patients were found to have AAV NAbs, and yet the gene therapy for them was still effective.

A larger gene therapy "vector" dose may be of significance in whether AAV Nab levels are treatable or refractory, observed Samelson-Jones and George. "Indeed, [Hemgenix] is administered at a 40-fold higher vector dose" than other AAV vectors studied. However, there is evidence to suggest that dose-limiting toxicities could be an issue with hemophilia gene therapies.

"Work is ongoing to develop methodologies to eradicate or avoid preexisting antibodies as well as prevent formation after vector administration," wrote Samelson-Jones and George.

Source: https://www.managedhealthcareexecutive.com/view/gene-therapies-for-hemophilia-promising-but-there-s-room-for-improvement-say-reviewers



THE YEAR AHEAD

15 - 17 September, 2023

Youth (18-30) event Wellington.

January, 2024

Youth (18-30) sailing Auckland.

1 -3 March, 2024

Teen and youth camp MERC, Auckland.

26 - 28 July, 2024

Women's wellness weekend Taupō.

06 - 09 February, 2025

National family camp Ngāruawāhia.

Visit www.haemophilia.org.nz for more information on bleeding disorders, HFNZ News, and past issues of Bloodline

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