Haemophilia New Zealand

Bloodline

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Trick or Treat(ment)?

Contents

Cover photo by **Hugo Gong**

Editor **Deon York**

Contributing Editor **Hugo Gong**

With thanks all contributors and to the Hive Creative for design and layout.

H **Word**

Tēnā koutou,

As we look back on 2025, there's a lot to celebrate and acknowledge. The year began with an over-subscribed National Families Camp in Ngāruawāhia, which brought together many of the newest members of our bleeding disorders community. We also marked the official opening of our new Wellington office, hosted a successful wellness weekend, and continued the period products pilot. Across the motu, branch activities have seen a resurgence thanks to the incredible efforts of our staff and volunteers working together to strengthen connections.

On the global stage, HNZ maintained a strong presence in the international bleeding disorders community. We were represented at both the WFH Comprehensive Care Summit and the WFH Global Forum on Safety and Supply, and continued our twinning projects in Pakistan and Fiji. A milestone worth noting: testing for the diagnosis of bleeding disorders took place in a Fiji hospital for the first time. You'll find more details from the CCS and Global Forum in this edition of Bloodline.

This year, we also farewelled two valued board members: Tracy Nyhan and Catriona Gordon. Tracy has long been a cornerstone of the central region and returns to her role there after a brief but impactful time on the board. Catriona's contribution spans nearly 18 years, much of it as vice-chair and safety and supply officer. Her commitment to centring on patients and families in decision-making has been unwavering, and her expertise and lived experience have shaped HNZ in meaningful ways.

As we thank those departing, we also welcome new faces. Lauren Phillips rejoins as vice-chair after a well-deserved break, bringing her global leadership in advocating for people with von Willebrand's and for better understanding of how bleeding disorders affect women and girls. Neville-James Reedy joins as Piritoto delegate, offering his unique perspective and experience. Ashley Fowlie and Conor Clerkin round out the new additions: Ashley continues her key role in the central region committee, while Conor brings fresh energy as someone new to the bleeding disorders community. Alongside continuing members Karl Archibald (treasurer), Tineke Maoate (board member), and Connor McCone (youth delegate), this is an engaged and passionate team committed to delivering the best for our community. It's a privilege to work alongside all of you.

There's plenty to look forward to in the coming year. In January, we kick things off with the Teen and Youth Camp, a great opportunity for young people to connect, learn, and have fun. In April, the global bleeding disorders community will gather for the WFH World Congress, and we're excited to see strong representation from Aotearoa. Later in the year brings the Women's Weekend, continuing our focus on supporting women and girls with bleeding disorders. Keep an eye out for updates and events from Youth, Piritoto, and your regional branches – there will be plenty of opportunities to connect and get involved throughout 2026.

I hope you all enjoy a safe and restful summer break, and I look forward to seeing you at events throughout 2026.

Mauri ora,
Hemirau Waretini
Chair

CE **Update**

What a packed year 2025 has been for our organisation and our community! We began the year with a new families camp, headed towards the middle of the year with the office officially opening in Wellington, and capped it off with a buoyant wellness weekend.

While I have always been a part of the community, 2025 represents to me one year officially as chief executive of HNZ. I am proud to be continuously associated with the people that make this mighty organisation. I remain motivated by your stories, living your lives as fully as you can despite the challenges bleeding disorders can bring.

I was both excited and delighted to attend the wellness weekend and (re)connect with those who attended. You can read more about my impressions and reflections of Wellness Weekend 2025 held in Wellington on page 4. You will also find Phil's summary of the weekend on page 9.

I recently attended both the Australian Bleeding Disorders Conference and, with Hemi Waretini, the World Federation of Hemophilia Global Forum. I detail information relevant to our community shared at these fora on pages 11 and 13. There is a lot to look forward to when it comes to advances in treatment for people with bleeding disorders. You will also find Hemi's impressions of Global Forum on page 7.

2026 promises to be full of fun and opportunities for our members, including the Teen and Youth Camp in January in Auckland, and, also in Auckland, the Women's Wellness Weekend in October 2026.

However you celebrate over the December-January period, I wish you time for reflection, relaxation, and, of course, celebration. I look forward to working with you all in the coming year.

Deon York
Chief Executive

Trick or Treat(ment)? **Wellness Weekend 2025**

From 31 October to 2 November, around 60 members gathered in Wellington to focus on health, wellness, and connection. How was the experience, and what does trick or treatment have to do with our members? I reflect on this weekend and what it shows about our community by particularly focusing on the main theme of the wellness weekend this year.

By **Deon York** Photographs by **Hugo Gong**

LEFT: Member and staff alter egos were on vivid display. RIGHT: Participants keenly listened at the workshops.

The wellness weekend this year took place on 31 October. For those who are Halloween-inclined, this date is that costume and candy-fuelled event of the year. On quick glance, the only commonality Halloween and Haemophilia share is the initials 'Ha', but let's look a little deeper. The common phrase in Halloween is 'trick or treat'. Trick or treat(ment) is also important to those with bleeding disorders and unsurprisingly, the main theme of the wellness weekend. In both cases, costumes can often mask what is going on under the surface, and blood features, although one blood link is real, while the other is hopefully a trick.

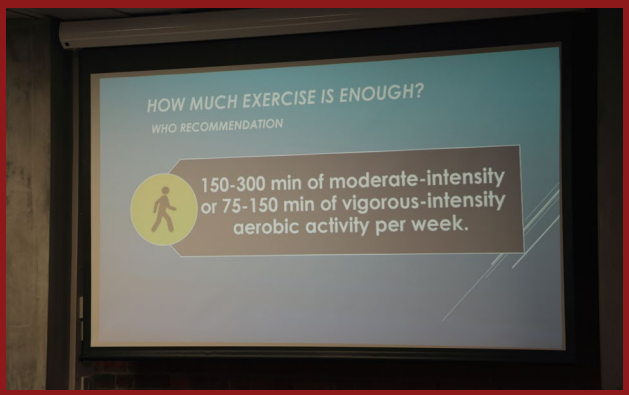
Some staff and members passionately embraced this theme with outreach workers transforming themselves to witches and other 'scary' creatures, and members becoming warlocks and bloodthirsty characters. I was never in any doubt of some of the alter egos on display, and others were an unexpected transformation.

The weekend focused on ways we can trick our mind and body to improve our wellbeing, while not forgetting that treatment is also part of looking after ourselves. It is equally essential to treat ourselves from time-to-time, and the weekend ensured that there was time to do just that. With discussions on wide-ranging topics, such as physiotherapy and keeping active, genetic diagnosis, relationships and sexuality, along with ageing, there was something for every attendee.

I was so inspired by our member Willy Tekira's talk with Veronique (Vee) Beets (Wellington Haemophilia Centre physiotherapist). Willy explained the transformation in his life because of starting and maintaining an active and healthy lifestyle. Before this journey, by his own admission, Willy was once in poor health, weighed a lot more, which put a lot more pressure on his joints. Other members of the 'master's group' who meet regularly and exercise together came along to support Willy and Vee. They presented a beautiful bouquet to Vee as a token of appreciation. Willy is such a powerful example of treating one's body and mind well – through taking regular treatment, and remaining active and healthy – thereby 'tricking' one's physical and mental wellbeing to achieve better health outcomes. As Vee pointed out, if Willy, with his sheer dedication and perseverance, was able to achieve such an impressive transformation, there is no excuse for the rest of us. There was obvious and sometimes uncomfortable 'chair shifting' when we all reflected on how much exercise we do. Willy, what an inspiration you truly are!

Unsurprisingly, BJ delivered a no holes barred presentation on ageing, followed by a Q & A on day two. If you wanted to know about ageing, sex, Scottish satire, and a whole range of topics, BJ certainly did not disappoint. Not enough words are available to convey the multitude of topics covered by our nurse specialist extraordinaire. Thank you, BJ!

Vicky Hernández (psychologist and sexologist) shared her insights about various facets of relationships, intimacy, and well-being in bleeding disorders. Vicky had long agreed to support our weekend, but due to unforeseen circumstances she joined us virtually from Argentina. This made her commitment even more special. Virtual presentations, especially those across different time zones, often pose a multitude of challenges. Despite that, Vicky's presentation was filled with interactive elements as well as group discussions and reflection. Our immense gratitude for the precious time Vicky devoted to being with us twice, and in grappling with a different time zone.



LEFT: Willy Tekira encourages us all to get active with support from Vee Beets, physiotherapist at the Wellington Haemophilia Centre.

TOP RIGHT: 'Hola' from and to Vicki Hernandez, psychologist from Argentina.

BOTTOM RIGHT: How much exercise is enough?

LEFT: A merry crew at the Wētā Workshop tour.
RIGHT: Vee presented with flowers by Ana on behalf of the Wellington master's exercise group.

Angela Seaton also presented options for genetic counselling. This was delivered in an accessible and relevant way to our members. A big thank you for giving up some of your weekend to be with us, Angela.

The wellness weekend was not about treatment for our physical and mental wellbeing only; attendees were also in for a treat, notably Wētā Workshop, Te Papa, and some exploration of what Wellington on beautiful sunny days had to offer. For the Wētā-goers, we were thoroughly entertained by the guides at Wētā Workshop who took us through the many wonders of film and special effects. Had we been there earlier, I would have definitely come up with a convincing Halloween costume. We saw a Sam Neill that has been thrown off a cliff, a giant bunny suit from The Killian Curse, and, of course, some weaponry from the classic Lord of the Rings Trilogy. Fortunately, the only 'precious' character on display was Gollum – we are not known to be a precious community by design.

Our annual general meeting also took place over the wellness weekend. We sadly bid farewell to Catriona Gordon as vice-chair of HNZ. She served the board for around 17 years. In recognising Catriona, I commented that her story epitomises who we are as a community. On a personal note, Catriona was always there with me during my time as chair as a wise counsel with lots of fun and empathy to match. I will definitely miss seeing you on the board but know that you cannot escape so easily as we will retain you as legal counsel. Hemi also recognised the departure of Tracy Nyhan on the board.

I have appreciated the time that Tracy has given to the board and equally know that Tracy remains a part of the community regardless of the time on the board concluding.

Having spent all this weekend with our community, what does it show me? I saw a diverse group who all had unique, powerful stories to share. I felt a positive atmosphere and a strong willingness of all to show up for both the education and the fun. I noted that the unwavering need to connect and share our experiences remains dear to our members. The next step is to understand what impact this event has had beyond the weekend itself. For those in attendance, you can expect follow-up from your trustee witch or goblin (outreach worker).

Last, certainly not least, a heartfelt thank you to everyone who made this weekend memorable and a success.

Afterword: They say you can't beat Wellington on a good day, which has been hard to come by this year. Despite that, Wellington was bathed in sunlight and warmth throughout the wellness weekend. We couldn't have asked for more.

WFH Global Forum 2025: **Driving Equity and Innovation** in Bleeding Disorder Care

The World Federation of Hemophilia (WFH) Global Forum 2025 brought together clinicians, researchers, and patient advocates from around the world to tackle some of the biggest challenges in bleeding disorder care. The conversations were inspiring and eye-opening, highlighting inequities in women's health, the need for inclusive research, and the exciting – but complex – future of emerging therapies.

By **Hemirau Waretini**

BIAS AND GAPS IN WOMEN'S CARE

Women and girls with bleeding disorders (WGBDs) still face major barriers to diagnosis and treatment. Iron deficiency affects over 2 billion people globally and hits women and children hardest. Menstruation is often stigmatized or brushed off as 'normal,' delaying recognition of underlying bleeding disorders.

- 40% of females aged 12-21 are iron deficient, yet routine screening is rare.
- Reference ranges for hemoglobin and ferritin often normalize iron deficiency, hiding the problem.
- In a UK survey of 11,000 women, half felt their symptoms were dismissed by healthcare providers.

Experts called for a rethink of what 'normal' menstrual bleeding looks like, better diagnostic standards, and structural changes to eliminate gender bias. Heavy menstrual bleeding (HMB) and postpartum hemorrhage (PPH) were flagged as critical issues – 95% of women with VWD experience HMB, yet 70% think it's normal.

INCLUDING WOMEN IN CLINICAL TRIALS

Despite years of advocacy, women still make up only 40% of clinical trial participants, a figure unchanged for two decades. For WGBDs, exclusion criteria often ignore gender-specific symptoms like menorrhagia, raising questions about trial relevance.

- Recent trials, like FRONTIER 2, showed that including women didn't compromise safety or efficacy.
- Four women participated, including two of reproductive age and one with inhibitors.
- Their outcomes mirrored those of male participants.

The takeaway? Design trials around symptoms, not gender. The American Society of haematology is working on a roadmap to embed diversity, equity, and inclusion into haematology trials – a promising sign of change.

The FRONTIER 2 trial was a Phase III study evaluating Mim8, a bispecific antibody mimicking factor VIII activity, in adolescents and adults with haemophilia A – with or without inhibitors. Participants received weekly or monthly Mim8 or continued standard therapy. Results showed up to 98% reduction in treated bleeds and significant improvements in quality of life. Importantly, women were included to ensure gender inclusivity.

VON WILLEBRAND DISEASE (VWD): STILL UNDERDIAGNOSED

VWD is the most common inherited bleeding disorder, yet it's widely underdiagnosed. Fewer than 1% of women with abnormal uterine bleeding are assessed for VWD, even though it can severely impact quality of life.

- Complex diagnostic tests.
- Limited awareness among healthcare providers.
- Structural barriers to care and prophylaxis.

Women with VWD face 4–5 times higher risk of postpartum haemorrhage, making proactive management essential.

ARE WE READY FOR REBALANCING AGENTS?

Next-generation therapies like rebalancing agents are changing haemophilia care. These treatments restore balance between clotting and anticoagulant factors rather than replacing missing clotting factors. Examples include anti-TFPI agents (concizumab, marstacimab) and siRNA therapies (fitusiran). They aim to reduce bleeding risk and bypass inhibitors but require vigilance for thrombosis risk.

Rebalancing agents work by reducing natural anticoagulants or enhancing procoagulant activity, allowing sufficient thrombin generation for clot formation. This approach is particularly useful for patients with inhibitors or those who do not respond well to factor replacement therapy.

What do Concizumab, Fitusiran, and Marstacimab treat?

- Concizumab: For haemophilia A or B patients, with or without inhibitors, who need prophylaxis. Works by inhibiting TFPI.
- Fitusiran: For haemophilia A or B patients, including those with inhibitors. Uses siRNA to reduce antithrombin.
- Marstacimab: For haemophilia A or B patients without inhibitors. Targets TFPI for prophylactic use.

Regulatory Journey: Licensing Status Around the Globe

Concizumab (Alhemo):

- Canada: Approved March 2023.
- Australia: Approved July 2023.
- European Union: Approved December 2024 after CHMP endorsement.
- United States: FDA approval December 2024 for haemophilia A & B with inhibitors; expanded July 2025 to include those without inhibitors.

Fitusiran (Qfitlia):

- United States: FDA approved March 28, 2025 for routine prophylaxis in haemophilia A or B, with or without inhibitors.
- Other Regions: Submissions pending; no major approvals announced yet.

Marstacimab (Hympavzi):

- United States & European Union: Approved in 2025 for prophylactic treatment in haemophilia A or B, with or without inhibitors.
- First once-weekly subcutaneous dosing option; supported by Phase 3 BASIS trial showing 93% reduction in treated bleeds.

HNZ Wellness Weekend **2025**

On Halloween, 31 October, a large group of members and staff met at the Naumi Studios in Wellington for the 2025 wellness weekend. This event is a fantastic opportunity for members aged 18 and over and their partners to get together, reconnect, and learn more about living with a bleeding disorder.

Text and photographs by **Phil Constable**

The event started with dinner and a welcome from CE Deon York, followed by a wonderful ice-breaker exercise run by Iona Kahu. This was a fantastic way for all the attendees to learn a bit more about each other and to mix and mingle before some of the education sessions that were to follow. It was lovely to see people who already knew each other reconnecting, and newer members getting to know others for the first time.

On Saturday we started with an education session from psychologist and sexologist Vicky Hernández, who spoke about relationships, intimacy, and well-being in bleeding disorders. We then had sessions that covered from birth to end of life. First up embryologist Angela Seaton spoke about genetic counselling, then physiotherapist Veronique Beets talked about exercise and movement, and finally nurse specialist BJ Ramsay spoke about ageing and bleeding disorders.

These were very informative sessions, and we appreciate the time the presenters put into them.

Saturday afternoon was put aside for outings. A large group went to Wētā Workshop, where they enjoyed a guided tour of the amazing creations. Another group visited our national museum, Te Papa Tongarewa. This was also a good opportunity for some to have time to themselves, enjoying the hotel and the sights of Cuba Street.

In the evening the whole group enjoyed dinner out. This was a lovely occasion, with good food and good company. Thanks to the team at Monsoon Poon for looking after us.

On Sunday morning we started with the HNZ AGM. This is always a wonderful opportunity for members to actively participate in the governance of our organisation. At this meeting we were able to welcome two new board members and appoint a new vice-chair. We also paid tribute to long serving board member and Vice-Chair Catriona Gordon. You can read more about the new board from page 23 to 30.

The day continued with some breakout sessions for people with haemophilia, von Willebrand's, other platelet disorders, and partners. These were very fruitful sessions, which could have gone on much longer. These sessions were followed by an ask-me-anything session featuring Deon, nurse specialist BJ, and psychologist Vicky.

Feedback about this weekend was very positive. Members enjoyed the opportunity to reconnect with friends from across the motu, as well as meeting new people with shared experiences. They also reflected on benefits from the education sessions and went away with ideas and plans for changes to improve their lives.

A big thanks has to go to HNZ staff for organising another fantastic event.

We would like to thank Sanofi for kindly sponsoring part of this event.

Reflections on **Australian Bleeding Disorders Conference**

With incoming president of Haemophilia Foundation Australia Alan Dursun (left) and outgoing president Gavin Finkelstein (right).

From 16 to 18 October, Haemophilia Foundation Australia (HFA) hosted the Australian Bleeding Disorders Conference in Brisbane. As the name suggests, the conference was to discuss *all* bleeding disorders with a focus on teamwork and 'pushing the boundaries'.

By **Deon York** Photographs: Supplied

This biennial conference is an important occasion for the bleeding disorder community in Australia, as all state and territory bleeding disorder organisations converge to socialise and learn together as well as from each other. The program offered sessions on family, women, lifestyle, youth, ageing, rare bleeding disorders, and advances in therapy. It brought together patients, health professionals, researchers, and policy makers. While naturally focusing on the care landscape in Australia, New Zealand shares many similarities in the delivery of care, community supporting one another, and the impact of advances in treatment on care in the future.

Professor Cedric Hermans provided an insightful overview of the new treatment landscape of bleeding disorders. I share my interpretation of what I heard at this recent conference.

Treatment for haemophilia has undergone a revolution in recent years. Factor replacement therapy is now considered 'classic' treatment in resource-rich countries (i.e. FVIII and FIX infusions). This classic treatment historically brought challenges in IV administration, short half-life, and immunogenicity (risk of inhibitors).

The 'classic' therapy in the treatment of bleeding disorders still has its place, but we are now witnessing even longer half-life classics, 'non replacement' therapies, ultra-long-acting therapies, and gene therapy. This evolution of treatment takes us from preventing bleeds to what is increasingly heard as a 'haemophilia free mind'. This is leading to new ambitions for bleeding disorder treatment including the goal of zero bleeds, minimal treatment burden, a 'normal' life, and even a cure. Ambition is also shifting from taking severe haemophilia, for

example, and effectively transforming the diagnosis to mild to the ambition of normal haemostatis. Gene therapy also promises *cure* for some affected by haemophilia A and haemophilia B. This statement will be made with more certainty as longer-term surveillance data becomes available.

Turning to some of the specific treatments: Modified recombinant FVIII and FIX with reduced clearance and a longer half-life mean fewer intravenous injections, higher trough levels, less treatment burden, and fewer bleeds. There are also ultra-long and high-sustained activity FVIII therapies with the freedom of a once-a-week infusion. While for haemophilia A, New Zealand has access to Hemlibra, this has not completely eliminated the need to use intravenous FVIII and certainly has not eliminated the need for FIX, so it is important we also remain aware of these therapies.

By now, we are aware of emicizumab (Hemlibra), which is not FVIII but a so-called 'bispecific antibody' that mimics the action of FVIII in coagulation. It represented a major step forward for our community with its subcutaneous administration, longer half-life, and elimination of inhibitors. Unsurprisingly, there are other such therapies in development. Mim8 (Novo Nordisk) and NXT007 (Chugai/Roche) are a couple of examples. Those two agents result in higher thrombin generation than emicizumab. Intriguingly, a potential oral treatment for haemophilia A is at the trial stage (Inno8). Subcutaneous therapies for the treatment of both haemophilia A and B exist, namely concizumab, marstacimab, and fitusiran. The dosing regimen of concizumab has been adjusted due to thrombotic events, marstacimab has no reported thrombotic events to date, and when fitusiran was at phase three clinical trials, adverse events led to a new dosing regimen to maintain levels of 15-35%. When it comes to these therapies, Professor Hermans noted that there are multiple strengths and numerous limitations; notably, they all create stable thrombin generation (independent of FVIII/IX), long half-lives, no cross-reactivity with FVIII/IX inhibitors, and are convenient. The flipside is thrombotic risk has been observed, a cautious approach must be taken in the management of trauma and surgery, and the superiority to other prophylactic options remains to be demonstrated.

Hermans also introduced *aptamers*. These are small single-stranded DNA or RNA molecules that bind to a specific target. They work like an antibody but are more stable, have longer shelf lives, have an animal free production line, and are easier to produce than antibodies. The BT200 aptamer has been shown to reduce clearance of the VWF/FVIII complex, resulting in a transient increase in plasma levels of both proteins. Among those participating in the trial, VWF levels were increased 3-4-fold, and in FVIII, 2-3-fold.

Gene therapy for both FVIII and FIX deficiency is available. If previously exposed to the viral vector (AAV), this therapy cannot be used. There is a frequent need for immunosuppression for FVIII gene therapy, and a less frequent need for FIX. Gene therapy offers the prospect of a 'haemophilia free-mind'.

Evidently, following many years of using similar therapies for the treatment of bleeding disorders, the explosion of research and development continues. While living with a bleeding disorder presents challenges, we are comparatively fortunate to be living at a time when so many therapies are undergoing clinical trials or have already been licensed for use. So long as there is access to these therapies, our community will continue to grow healthier and stronger.

A heartfelt thanks to Natasha Coco, Executive Director, HFA and the entire team for generously supporting my attendance. It has meant an opportunity to share this valuable information with all HNZ members.

LEFT TO RIGHT: Team NZ at the conference; With Natasha Coco, Executive Director, Haemophilia Foundation Australia; Participants gather for conference opening.

Global Forum 2025

The World Federation of Hemophilia (WFH) Global Forum took place between 13 and 14 November in Montreal, the headquarters of WFH. This year's forum focused on advances in therapies for bleeding disorders and associated areas of importance to our community. This includes developments in global safety and surveillance, rebalancing agents, care for von Willebrand Disease, gene therapy, women and girls with bleeding disorders as well as emerging therapies and technologies. Thanks to a travel grant, I was able to attend and report back on my impressions of this year's forum.

By **Deon York** Photographs: Supplied

I focus on areas of relevance to our community and issues that potentially impact us sooner. For instance, while gene therapy was discussed and it is important for us to be informed about is progress, these therapies are far off in our context. Of course, HNZ continues to keep up to date on developments in this area.

Hemi and Deon with Rana Saifi, Regional Manager for the Middle East WFH and Emna Goudier, VP NMO WFH.

LEFT: Forum participants learning about new developments in care.
RIGHT: Brian O'Mahony, CEO of Irish Haemophilia Society, presents on product tendering processes.

SAFETY AND SURVEILLANCE

Changes to the healthcare system in the United States may seem far away, but each decision made by US agencies can impact products globally, including product safety. Agencies such as the CDC, NIH, and FDA make decisions that can impact availability of products in our health system, too.

While there are no known threats in our blood supply, a continuous need to be vigilant remains. The important message I took away from this session was that while we are a relatively isolated country, we are naturally affected by global realities. There will always be 'TNV' (the next virus), which is why two quotes stuck in my mind by Steven Grossman: First, 'While the pathogen minefield becomes broader and more treacherous, the regulatory landscape has become less predictable and reliable.' Second, 'We must preserve and expand the systems created in response to the disasters of the 1980s'.

BIAS AND GAPS IN WOMEN'S CARE

Dr Angela Weyand highlighted the impact of sexism and the stigma of menstruation on the underdiagnosis of iron deficiency in women. Iron deficiency impacts 2 billion people. Women and children are more heavily impacted, and iron deficiency is linked to increased mortality and morbidity. Put differently, with or without a bleeding disorder, this is a major global health challenge. The World Health Organization continues to frame iron deficiency as a nutritional issue; in fact, most pregnant females and 40% of females aged 12-21 experience iron deficiency.

Many health providers are likely to attribute symptoms to psychosocial issues. Many are informed that symptoms are to be 'expected and accepted' and many reference ranges for haemoglobin and ferritin normalise iron deficiency. Dr Weyand emphasised that this is a *draconian* approach to caring for women with iron deficiency.

Dr Stephanie Seremetis discussed the inclusion of women in clinical trials. Only 41% of patients included in clinical trials are women. Dr Seremetis noted that trials need to reflect real world settings and the reality of bleeding disorders.

She also raised the principle of 'treat first and investigate later'. This often applies to treating a bleed for haemophilia, so this begs the question as to why it cannot apply to treating women and girls with bleeding disorders. After all, according to CDC data from 2012 to 2020 around 1 in 5 women with bleeding symptoms effectively have mild haemophilia. Current options for treating women and girls with bleeding disorders include DDAVP, plasma-derived VWD factor, tranexamic acid, and oral contraceptives.

Dr Michelle Sholzberg pointed out that since women do not often discuss periods, the impact on quality of life is often 'normalised'. With normalisation, many women see the bleeding as normal due to other experiences within the family, often resulting in diagnostic delay and lack of recognition.

Hemi and Deon with Mariana Battazza; Barry Flynn, VP Finance WFH; and Ekawat Suwantaroj, WFH board member.

VON WILLEBRAND DISEASE

Dr David Lillicrap explored future treatments for VWD, presenting several novel therapies currently in development. Emerging therapies include recombinant VWD factor, which may benefit challenging mucosal bleeding, and the use of Hemlibra in severe VWD with FVIII less than 15% where recurrent joint bleeds are a problem.

Other therapies in development include prolonged VWF half-life, with one in development increasing thrombin levels.

Professor Emna Gouider argued that comprehensive testing for VWD is often impractical in low- and middle-income countries and promoted the concept of prioritising a few key assays for diagnosis.

REBALANCING AGENTS

A lot is currently happening around 'rebalancing agents'. Rebalancing agents are therapies that correct haemostatis but do not replace the missing protein (for instance, FVIII). Examples include anti-tissue factor pathway inhibitors like concizumab and marstacimab, and anti-thrombin inhibitors such as fitusiran.

Professor Mike Makris focused on the thrombotic risks associated with these rebalancing agents, suggesting a higher risk in older patients. He compared the rates of thrombosis in clinical trials to those having regular treatment. He suggested that as these products are progressively introduced, there is a need for careful monitoring and transparent reporting, particularly as they are applied in the real world, and when given to older patients. That said, these therapies all show significant promise in improving annual bleed rates and outcomes for patients.

AI AND ITS POTENTIAL IN BLEEDING DISORDER CARE

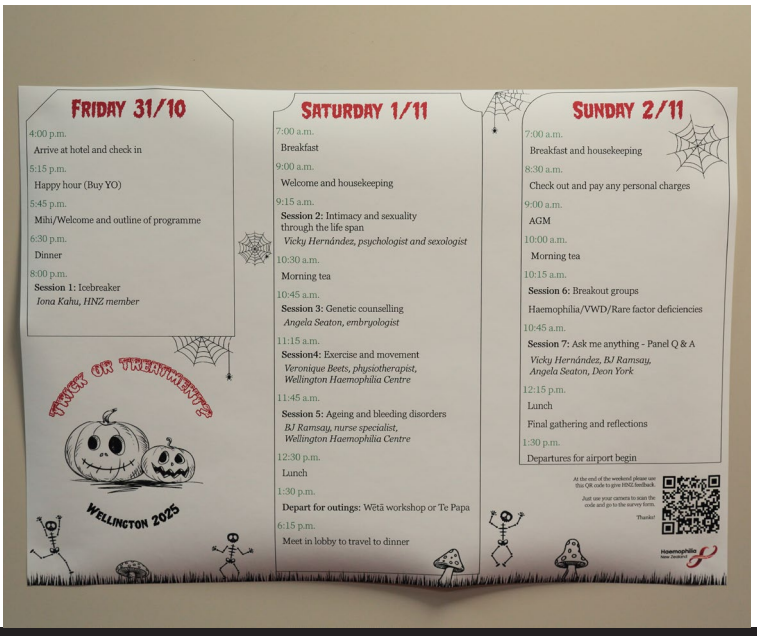
Professor Cedric Hermans gave an interesting and impassioned talk on the possibilities of AI. He noted upfront that AI gives an impression of intelligence, but this is an illusion based on statistical coherence. In other words, AI cannot simply replace people.

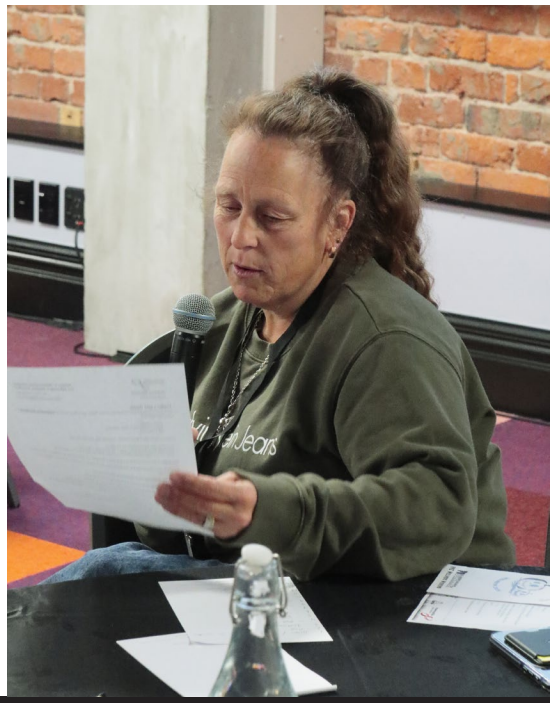
There are ways that AI already assists in the treatment of bleeding disorders such as joint imaging, research on genes and proteins, robotic systems in surgery, and even by way of multilingual chatbots. AI is also transforming medical education, and education in general. The future of AI, and its place in treatment raises the question: Will people with bleeding disorders ultimately become 'data coordinators'? This would require careful thought on the ethics of self-management in a way not seen before.

AI technology was used to summarise the entire global forum. While this helps with reporting back, it is interesting to note that the summaries generated are far more general than the insights of the speakers. This indicates to me that while AI is a helpful tool, we must appreciate it as such.

A Welly Wellness Weekend to Remember

Photographs by **Hugo Gong**






NOT MY CIRCUS


Exit
For access to Dunlop Terrace

Meet the **HNZ Board**

The Haemophilia NZ (HNZ) Board is elected by the membership to serve the NZ bleeding disorders community, ensuring that the organisation continues to make an impact and improve the lives of people affected by inherited and acquired bleeding disorders across Aotearoa New Zealand.

By **Phil Constable** Photographs by **Hugo Gong**

In November 2024 the composition of the HNZ Board changed. Long-standing Chair Deon York stood down and was subsequently appointed as the new HNZ chief executive. As such, previous Treasurer Hemirau Waretini assumed the role of chair, and former Vice-Chair Karl Archibald took the position vacated by Hemi, leaving Catriona Gordon as the sole vice-chair.

At the AGM in 2025, long serving Vice-Chair Catriona Gordon stood down after 18 years, as did board member Tracy Nyhan. In their place we saw former board member Lauren Phillips step up to become vice-chair, and Neville-James Reedy became the Piritoto representative, while Central Chair Ashley Fowlie and member Conor Clerkin joined youth representative Connor McCone and board member Tineke Maoate around the table. Along with CE Deon York, an ex officio member, we now have a full complement. Together, these HNZ members work to improve the lives of people and whānau affected by bleeding disorders in Aotearoa New Zealand by promoting excellence in care, advocacy, support, and education.

Alongside these role changes came an update to the HNZ constitution. The last time HNZ updated the constitution was in 1997. Many of the changes were new requirements under the Incorporated Societies Act 2022, and we took the opportunity to go further, making some changes to the governance structure, including implementing term limits for board members, and extending terms to four years. We've reduced the administrative load on the regional committees and tidied up the procedures around meetings of HNZ.

The changes to the constitution reflect our new name, Haemophilia New Zealand. You can read the current HNZ constitution on our website.

So, who are the people leading HNZ into the future?

Chair: **Hemirau Waretini**

Hemirau has been involved with HNZ from a young age and has supported national workshops, conferences and camps. Hemirau has been an active member of the Northern region and worked with others to lead subcommittees involving substantial work in the areas of Māori engagement and youth development. He was previously involved in the HNZ's twinning with the Nepal Haemophilia Society, leading delivery of workshops aimed at developing the capability of youth members of Nepal's bleeding disorders patient organisation. Hemirau works in the public sector.

Vice-Chair: **Lauren Phillips**

Lauren is a von Willebrand's patient with 20 years of HNZ involvement, having served on the board as both a youth delegate and regional delegate. She is also a member of the World Federation of Hemophilia's Women and Girls with Bleeding Disorders Committee.

She is currently involved in a pilot project providing period products to members, reflecting her commitment to addressing bleeding disorder impacts on women and girls nationally and internationally.

Treasurer: **Karl Archibald**

Karl is based in Christchurch and has severe Haemophilia A as well as a secondary condition, Classical Ehlers-Danlos Syndrome (cEDS).

Karl has been involved with HNZ from 1996 when he attended his first camp. From 2005 he has been an active member of the Southern Committee and a board member since 2007. He has mentored and supported HNZ camps and activities and participated in international youth development and mentorship programmes such as SURO and AFFIRM. He has a particular interest in the future of Haemophilia NZ and how to best support members.

Youth representative:
Connor McCone

Living with severe haemophilia, Connor serves as the youth delegate on the board. Before embarking on his legal studies, he worked as a welder and fabricator. Connor is actively engaged in the World Federation of Hemophilia's Youth Twinning Programme in partnership with the Haemophilia Foundation of Pakistan. He looks forward to updating the members of HNZ on what is achieved during this partnership.

Piritoto representative:
Neville-James Reedy

Nev has been involved in HNZ for over 40 years, as a person with severe Haemophilia A. Originally from the East Coast of the North Island, Nev now lives in Invercargill and works as an audio engineer and programme facilitator at the Southern Institute of Technology. A native speaker of Te Reo Māori, Nev also fulfils the role of Tikanga Advisor for the School of Screen Arts in Southland.

Nev sees that the purpose of his role is to facilitate engagement and support for Polynesian and other ethnic minority groups in NZ. His goal is for us to challenge our personal limitations to build better relationship with ourselves, and so better serve the wider bleeding disorders community.

Board member: Ashley Fowlie

Ashley has been involved with HNZ for over 12 years and currently chairs the Central Region. As a registered mental health nurse with 5+ years' experience, she's passionate about supporting HNZ, advocating for women with bleeding disorders and von Willebrand's, and giving back to an organisation that's positively impacted her life.

Ashley has organised events, workshops, and education sessions, facilitating educational topics for the community. Her mental health expertise, leadership skills, and organisational abilities are valuable assets to support HNZ.

Board member: **Conor Clerkin**

Conor brings significant experience as a complaints manager and investigator (including with the Health and Disability Commissioner), with skills in critical analysis, sound decision-making, fairness, and collaboration. He is also an AMINZ accredited mediator.

As a parent of a child with severe haemophilia, he is motivated to contribute to HNZ's impact and help others benefit. He is interested in governance-level involvement to utilise his judgment and communication skills.

Board member: **Tineke Maoate**

Tineke and her husband both live with a bleeding disorder. They have seven children who also suffer from platelet disorders to different degrees.

Tineke enjoys working to support other members with bleeding disorders and sharing their challenges.

In her spare time Tineke works as a volunteer fire fighter, board of trustees' member for her children's school, and she and her husband foster children through Oranga Tamariki. She hopes to educate and support other families learning about their conditions.

Chief Executive: **Deon York**

Deon brings over 25 years of governance and leadership experience in health and community sectors, championing improved lives for people with haemophilia and inherited bleeding disorders. His bleeding disorders advocacy includes extensive board-level roles at HNZ, ten years as a World Federation of Hemophilia director (2010-2020), and ongoing WFH engagement.

Driven by creating patient-centred healthcare systems, Deon received the 2024 International Frank Schnabel Volunteer Award for his global training and advocacy contributions.

Meet the HNZ Board: Beyond the Surface

While you might know what our board does, how much do you know about our board members? The final HNZ board meeting this year took place at the end of November with newly elected members. We seized this opportunity, posed some quick-fire questions to them, and received some surprising or intriguing responses! We share them below (in alphabetical order according to first name).

By **Deon York** Photographs by **Hugo Gong**

WHAT IS YOUR FAVOURITE PART OF THE COUNTRY AND WHY?

Ashley: It would be difficult to pick one part, as I love exploring new places in New Zealand. However, I do enjoy a day out walking around the Esplanade Gardens with friends.

Connor: Central Otago. It is where I grew up.

Conor: Living in Wellington, I really like the Kāpiti Coast and Wairarapa because it's a lovely long trip to enjoy a drive and get away from the day to day.

Deon (ex officio): The East Coast of the North Island, which reminds me of my mother and grandfather and the South Island for the breathtaking scenery.

Hemi: Aotearoa has such a rich natural and cultural landscape that picking a single spot is difficult! Recently, my wife and I walked the Kepler track in Fiordland. Sunrise from Luxmore hut was stunning. Abel Tasman National Park has some of the most beautiful beaches anywhere in the world, and walking through the Central North Island's elevated landscape gives a unique sense of perspective and place in the landscape when looking north towards Taupo. I also have great memories of my early years growing up in the ruggedly beautiful Waitomo District. As an Aucklander for most of my life, I also have to put in a plug for our biggest city. Home to some of our best restaurants and cultural events, as well as wild west coast beaches, the Hauraki Gulf and its amazing wildlife, and the Gulf islands and Great Barrier providing opportunities for off-grid adventures.

Karl: I may by a little bias, having spent my formative years here and I am sure it would be hotly contested, however, this place is the centre in the Aotearoa crown. With so much on offer from the hills to the ocean and everywhere in between would have to be Nelson/Tasman.

Lauren: The West Coast of the South Island – the West Coast is the best coast! It's rugged, beautiful and wild. There's something about being surrounded by such large landscapes that makes you feel insignificant, which is really grounding.

Nev: Moerangi, Ōamaru (It has a special mauri and feel there).

Tineke: Northland, because of the beaches. It doesn't matter what the wind is doing as you can go to either the West or East Coast.

I GET EXCITED AND MOTIVATED WHEN ...

Ashley: Shopping for bargains, spending time with family and supporting our bleeding disorders community.

Connor: When I am doing something I am passionate about.

Conor: When I get to meet new people and help others in some way.

Deon: I can see progress and the hard work was worth it.

Hemi: I'm a part of a collaborative team making progress on meaningful work.

Karl: Seeing progress on shared goals and hitting the targets that we have set out to achieve.

Lauren: I get really excited about trying a new place to eat when we visit the city.

Nev: Writing or creating something new.

Tineke: When I'm helping others.

OUTSIDE OF WORK, I'M MOSTLY FOUND ...

Ashley: Going for walks and spending time with my best friend, Amy.

Connor: Studying and going gym.

Conor: Playing with my young son and having a cup of tea.

Deon: At the movies, at Yum Cha, or doing my best to relax.

Hemi: Swimming at my local beach, enjoying great food with friends and family, listening to live music, spending time in nature or struggling to keep my house plants alive.

Karl: Spending time with my wife and two children, working on the many evolving house projects and mountain biking.

Lauren: Outside of work, I'm most likely found at the swimming pool or park with the girls, and hopefully this summer getting out and about on some local tramps.

Nev: Writing and creating.

Tineke: Firefighting or developing our land, but to be honest, telling my husband what I want built or what I want done. I'm really good at delegating or supervising him.

WHAT IS YOUR MOST FAVOURITE EXERCISE, AND HOW OFTEN DO YOU EXERCISE?

Ashley: Weekly walks with my friend Amy and my dog Luna.

Connor: I enjoy swimming.

Conor: Running and swimming. Not so often in recent times!

Deon: I can't say I enjoy exercise, but I know it is important and walking as often as I can within my routine is probably the most exercise I do. If I am in an interesting place, I am likely to walk much further.

Hemi: Yoga, as often as possible. Swimming and hiking (mostly when the sun is shining).

Karl: I may have given this away earlier … mountain biking, we live a short ride from some great local trails, I try to head out 2-3 times a week.

Lauren: My favourite exercise is playing netball! I generally get to play once a week with summer and winter competitions though I may have to switch to walking netball soon! I have reset my attitude around exercise as I've gotten older and with the help of our awesome physio Vee, and now its less about intensity or perfection and more about consistency. I try to walk most places rather than driving so I can get some exercise in and also set a good example for my girls.

Nev: Push ups (but not often anymore).

Tineke: What's exercise?

WHAT BOOK, TV SHOW, OR MUSIC ARE YOU CURRENTLY READING, WATCHING, OR LISTENING TO?

Ashley: The Chicago Med and Chicago Fire series, and as we go into December, it's Christmas music.

Connor: I love to watch pretty much any type of sport.

Deon: I've recently watched The White Lotus. It's a sharp commentary on people and privilege.

Hemi: I'm re-reading The Name of the Wind by Patrick Rothfuss, a great adventure (even though readers have been waiting for the third book in the trilogy since 2011!). Listening to: one of my eclectic Spotify playlists (think anything from Khruangbin to REM to Salmonella Dub to Doechii or Angie Stone). Watching: Slow Horses.

Karl: Book: The Outsiders of Orkland, A first novel written by my good friend.
Available here:

TV Show: The Bear.
Music. I have recently discovered the 2 Johnnies. Self-described as Somewhere between a cultural phenomenon, your favourite aul fella sitting in the corner of the pub and a chipper that everyone loves.

Lauren: Lainey Wilson. I'm a big country music fan and super excited to be heading to her concert in February.

Nev: The Lost Book of Enki.

Tineke: Dyslexic so no reading. Reality TV as it makes me feel normal and not as dysfunctional. As for music, the good, the great, the best 70s, 80s, and 90s.

WHAT ARE THREE WORDS THAT BEST DESCRIBE YOU?

Ashley: Organised, caring, dedicated.

Conor: Friendly, sentimental, frugal.

Deon: Determined, open, positive.

Hemi: Friendly, curious, organised.

Karl: Do you mean right now?

Lauren: Caring, outgoing, busy.

Nev: A question for others, perhaps?

Tineke: Mad, chaotic, ADHD.

WHAT IS ONE THING ABOUT YOU THAT WOULD SURPRISE PEOPLE?

Ashley: I love Chrismas, giving to others and putting up Christmas decorations.

Connor: Before I was a student, I was a welder fabricator.

Conor: I stopped a marathon.

Deon: I learned how to speak three additional languages.

Hemi: I used to be a BodyBalance instructor at Les Mills.

Karl: That I have a family history of Haemophilia dating back to 1890, impacting 11 people across 5 generations.

Lauren: Nothing!

Nev: Discovering I have severe haemophilia A!

Tineke: I don't believe there is anything that would surprise anyone about me. I am always an open book.

WHAT DO YOU MOST WANT TO ACHIEVE FOR HNZ IN 2026?

Ashley: Another role I have is chair of the Central Region. I would love to see the committee grow and some new ideas for events to engage and support our members.

Connor: Continue to grow the youth group.

Conor: Better understand and help meet patient and whānau need.

Deon: I want to build on the momentum of 2025 and ensure that whatever we do, we are asking if this action or approach best supports our community.

Hemi: Continue the re-invigoration of our volunteer-led opportunities for connection in the community, and identifying and addressing unmet needs.

Karl: Maintain a strong service offering to our membership through tough financial times.

Lauren: I'm really keen to get the period project up and running, and run an online workshop with a haematologist or nurse to discuss periods!

Nev: Re-establishing Piritoto.

Tineke: Representation of our members and delivery of new information.

Comprehensive Care Summit Highlights

Bridging gaps in care: Physical health, gender equity, and innovation in bleeding disorders

The CCS discussions on haemophilia and related conditions were underpinned by recurring themes of lived experiences and scientific outcomes; they highlighted medical, cultural, systemic, and social challenges that shape patient outcomes. This session provided a multi-dimensional view of haemophilia care, focusing on physical activity, bone health, musculoskeletal challenges, and gender-specific issues. It emphasised the importance of proactive screening, equitable access to care, and education for women and girls with bleeding disorders (WGBD).

By **Karl Archibald** Photographs: Supplied

ACTIVITY AND BONE HEALTH: A FOUNDATIONAL CHALLENGE

The World Health Organization's guidance for older adults emphasises 150–300 minutes of moderate aerobic activity weekly, complemented by strength and balance exercises. For individuals with bleeding disorders, this recommendation is both essential and complicated. Walking alone offers limited benefit for bone density; resistance and weight-bearing exercises are far more effective. However, how do we safely integrate these exercises for those at risk of bleeds?

Bone health emerges as a critical vulnerability. Osteoporosis and osteopenia often result when bone breakdown outpaces rebuilding – a process accelerated by inactivity, hormonal changes, and bleeding episodes. Combined with musculoskeletal complications such as arthritis, sarcopenia, and chronic pain, the risk of frailty and falls increases dramatically. One observation that resonated was the 'fall cycle': a single fall can trigger fear, reducing activity, which then weakens muscles and balance, making another fall more likely. Breaking this cycle is a priority.

WOMEN AND GIRLS WITH BLEEDING DISORDERS: CLOSING THE EQUITY GAP

The conversation expanded into the experience of women and girls with bleeding disorders (WGBD). Cultural stigma, delayed diagnosis, and systemic bias persist. Many normalise heavy menstrual bleeding – 40% never seek care. They discussed that it is not uncommon to have experiences where historical sexism in medical practice means women often feel dismissed, and where clinical research has largely focused on men. Even today, trial designs and outcome measures frequently fail to account for female-specific realities, such as menstruation. The discussion emphasised: This is not just a gap; it is a call to action.

Solutions must go beyond medicine. Education and cultural competence are essential. Health professionals need training to understand how cultural factors influence symptom reporting and treatment decisions. Communities need stigma reduction campaigns. Women need tailored education to empower informed choices.

Innovation and access: A promising horizon

Despite these challenges, the future holds promise. Extended half-life factor therapies, gene therapy, and genetic screening are reshaping possibilities. Outreach programmes and proactive carrier screening can close diagnostic gaps, but access remains uneven especially in resource-limited settings like South Africa. There, resilience shines through mobile clinics and community health workers are making a tangible difference.

Prevention and lifestyle: Everyday interventions

Prevention strategies deserve emphasis. A balanced diet, combined with safe resistance exercises, can help maintain bone health. Mental health support and personalized treatment plans complete the picture. Patients are encouraged to stay engaged in their communities, advocate for better care, and adapt treatment to lifestyle needs.

Reflections and implications

These conversations highlighted that improving outcomes for people with bleeding disorders requires a holistic approach. Physical health, equitable access to care, cultural sensitivity, and innovation must converge. As comprehensive care evolves, the challenge is not only to advance science but also to ensure that progress reaches every patient regardless of gender, geography, or socioeconomic status.

'BEATING HAEMOPHILIA': A PERSONAL STORY

Advances in transplants and gene therapy are transforming haemophilia care, offering the possibility of life without bleeding disorders. However, these treatments often focus on the medical outcome, while the psychological and emotional aspects remain less discussed. Dr Ronald Mahomane spoke to the psychosocial challenges of transitioning from a lifelong condition to a new health reality. He shared his personal experience, discussed mental health considerations before and after treatment, and the long-term impact on identity, resilience, and quality of life, and the ongoing need for support systems beyond medical care.

The journey towards connection: Leap from haemophilia to hope

When Dr Ronald Mahomane, astrophysicist, person with haemophilia, gene therapy patient, stepped onto the stage, he didn't just share research; he shared his life. His presentation was a blend of science, philosophy, and deeply personal experience.

The haemophilia paradox

'The paradox? Both realities exist simultaneously on the same planet.'

He explained what he called *'The Haemophilia Paradox: A Relativity of Suffering'*. In high-income countries, haemophilia is largely a managed

condition, prophylaxis, synthetic clotting factors, and near-normal lifespans. In low-income nations, the same diagnosis can mean untreated bleeds, crippled joints, and early graves.

'Time is not universal … It bends, shifts, and stretches based on your frame of reference. For haemophilia, your frame of reference is your country.'

Growing up in South Africa, Ronald lived the harsh side of that paradox. He spoke candidly:

'The potential heartbeats we never feel … are those lost between how long we live, how long we learn, and how long we suffer.'

For someone below the poverty line, he calculated: Life Expectancy – Years of Education – Years in Pain = 14.5. Fourteen and a half years of meaningful life – that's all many get.

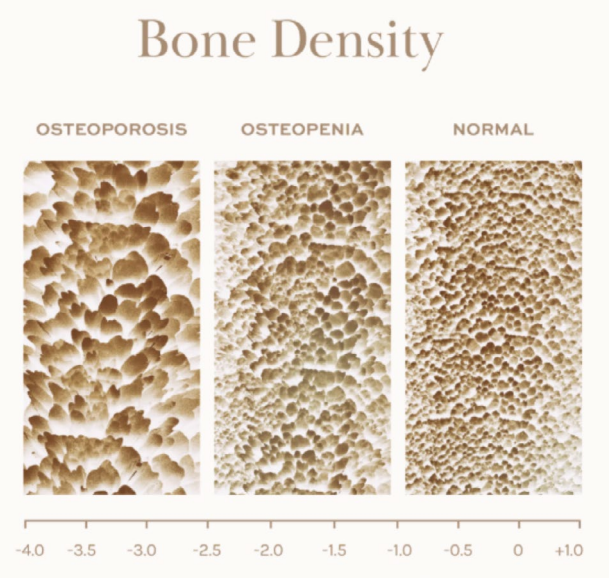
He described childhood marked by fear: every fall could mean a bleed; every bleed could mean a hospital visit that might not happen. Education was interrupted, dreams deferred. 'Pain became a language I spoke fluently,' he said.

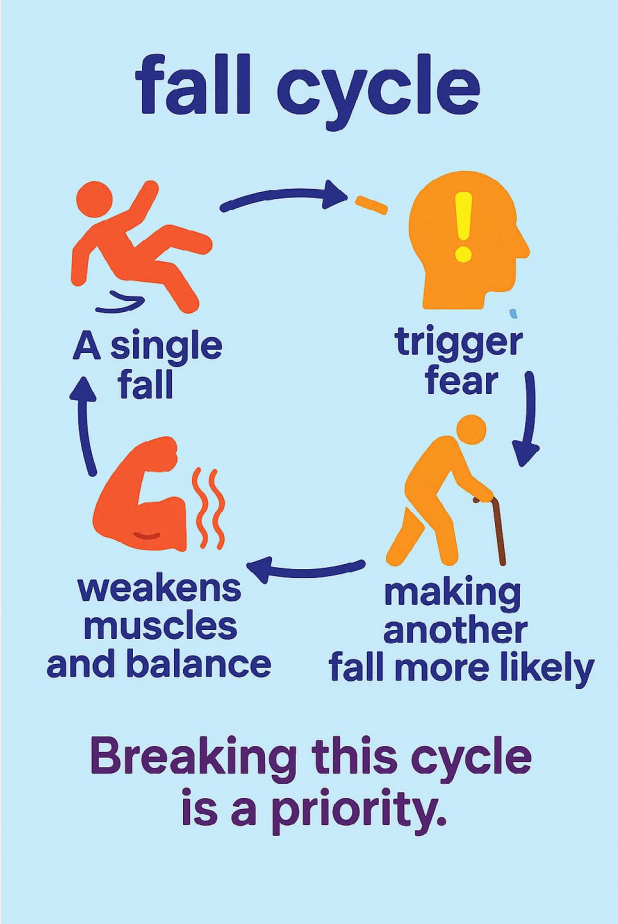
The turning point: Gene therapy

His journey changed with gene therapy. He described the process step by step:

- Screening: Genetic profiling, liver health checks, and viral vector compatibility.
- Infusion Day: *'It was surreal – the sterile room, the slow drip of hope into my veins.'*
- Waiting: Weeks of monitoring clotting factor levels, hoping for the numbers to rise. And they did. Slowly, then steadily. *'For the first time in my life, I could imagine a future without constant pain.'*

But he was honest:
'Gene therapy isn't perfect. It's expensive, not universally available, and its long-term effects are still being studied.'





Still, for Ronald, it was a leap toward freedom – a chance to live, learn, and hope.

Global disparities

Ronald didn't stop at his story, he zoomed out to the global picture.

- In high-income countries, comprehensive care and prophylaxis are standard.
- In upper-middle-income nations like South Africa, treatment is limited to major cities and often on-demand.
- In low-income countries, care is almost nonexistent.

He highlighted the stark contrast: *'In some places, haemophilia is a chronic condition. In others, it's a death sentence.'*

He spoke about the need for leapfrogging – where low-income regions bypass traditional, resource-heavy systems and adopt innovative, sustainable solutions like gene therapy and digital health platforms.

'Healthcare in low-income regions could leapfrog traditional systems,' Ronald said. *'But only if innovation is co-created, not imposed.'*

As his talk ended, two questions were posed by the facilitator that revealed another paradox: *'Clinicians, raise your hand if you would recommend gene therapy.'* Most hands went up.

'People with bleeding disorders, if you were given the option today, would you undertake gene therapy?' Most hands stayed down, the feeling is it is not quite there.

It is an intriguing how far gene therapy has come in recent years and for early adopters like Ronald it paves the way for further research and development; I am certainly looking forward to what the future holds.

Latest News

Compiled by **Phil Constable**

Preclinical data bring human trial launch closer for MGX-001

Primate study shows treatment restores clotting factor in hemophilia A.

By **Patricia Inácio, PhD** | November 25, 2025

Data from non-human primates suggest gene-editing therapy MGX-001 may be able to restore clinically meaningful levels of factor VIII (FVIII) after a single dose, supporting its potential as a one-time treatment for hemophilia A.

Developer Metagenomi expects to meet with health authorities later this year to develop a strategy for the therapy's development process and to file an investigational new drug application seeking approval for a clinical trial in late 2006.

"Our new data builds upon an earlier study demonstrating durable and stable FVIII activity in NHPs [non-human primates] over an approximately 19-month study, giving us confidence that our novel approach has the potential to be a curative, one-and-done treatment for patients suffering from hemophilia A," Jian Irish, PhD, president and CEO of Metagenomi, said in a company press release.

Hemophilia A is caused by mutations in the F8 gene, which provides instructions for making clotting factor VIII (FVIII). Without enough FVIII, the blood cannot clot normally, leading to frequent or prolonged bleeding episodes that may damage joints and tissues over time.

MGX-001 aims to correct this underlying problem by enabling the liver, the body's primary source of clotting factors, to produce its own functional FVIII.

'A potentially lifelong cure'

The treatment uses a two-part system: an engineered adeno-associated virus (AAV) delivers a working F8 gene, while lipid nanoparticles (LNPs) provide a guide RNA and a DNA-cutting enzyme, or nuclease, to insert that gene into a highly active region of the genome known as the albumin locus. Once inserted, the new gene may allow liver cells to make FVIII at levels high enough to prevent spontaneous bleeds.

Instead of temporarily replacing or mimicking the missing clotting factor, "MGX-001 enables endogenous production of FVIII for hemostatic regulation and restores the body's own ability to produce FVIII for a potentially lifelong cure," Irish said.

Researchers tested MGX-001 in 24 non-human primates. Animals received one of six AAV doses ranging from 5.0×10^{11} to 4.0×10^{13} vector genomes per kilogram (vg/kg), followed by an LNP dose of 0.2, 0.6, or 2.0 mg/kg. All animals received corticosteroids before both components.

Across the five highest AAV doses, animals reached therapeutic FVIII activity, defined as 50% to 150% of normal human FVIII levels. In one group, where the LNP dose was administered at 0.6 mg/kg and AAV doses varied between 1.6×10^{12} and 4×10^{13} vg/kg, the average FVIII activity ranged from 49% to 81% of normal. When the AAV dose was fixed at 5×10^{12} vg/kg and the LNP dose varied, FVIII activity ranged from 17% to 72%.

The proposed clinical dose – AAV 5×10^{12} vg/kg plus LNP 0.6 mg/kg – yielded an average FVIII level of 49%, with individual results ranging from 29.3% to 59.5%. No animals exceeded the upper safety limit of 150%, and no off-target genome edits were detected.

"We are highly encouraged by the dose range finding results observed in this study where we have seen clear dose-dependent activity across both the AAV and LNP components of MGX-001, resulting in therapeutically relevant FVIII activity in each animal treated in all but the lowest AAV dose," Irish said.

Aside from temporary liver-enzyme elevations at the highest LNP dose, MGX-001 was well tolerated.

Hematology consultant Glenn F. Pierce, MD, PhD, said the therapy could meaningfully expand options for people with hemophilia A. "The MGX-001 approach represents a potential paradigm shift for the treatment of hemophilia A patients who, even with currently approved therapies, are subject to rare but serious spontaneous bleeding events and must always ensure access to their treatment," Pierce said. "As a physician scientist, drug developer, and former hemophilia A patient myself, I can speak firsthand to the impact that a potential one-and-done curative treatment can have in enabling a new standard of life with a hemophilia-free mindset."

This article originally appeared on: https://hemophilianewstoday.com/news/preclinical-data-bring-human-trial-launch-closer-mgx-001/

Self-BAT can help screen for carriers of bleeding disorders, such as hemophilia

By **Sheila Jacobs** | 17 November 2025

The online Self-administered Bleeding Assessment Tool (Self-BAT) has been validated as a screening instrument for potential carriership of hemophilia, von Willebrand disease, and platelet function disorders.

A prospective study was conducted among patients referred to a tertiary hematology clinic located in Kingston, Ontario, Canada, for evaluation of a possible bleeding disorder. Findings from the study were published recently in Haemophilia.

In 2010, the International Society on Thrombosis and Haemostasis (ISTH) developed the ISTH-Bleeding Assessment Tool (ISTH-BAT). The tool was designed to be administered by healthcare professionals, thus rendering it inaccessible for widespread public use. It includes a series of questions and associated scores that are intended to "standardize quantitative reporting of bleeding symptoms."

Following this, the Self-BAT was developed in 2015 and originally designed in a paper format to be used in primary care settings by a primary care provider. In this regard, patients were able to establish their bleeding scores independently using layperson terminology.

The opportunity for patients to have their symptoms evaluated with the paper Self-BAT or ISTH-BAT was limited, however, because a high percentage of individuals do not have a primary care provider. To address this issue, the researchers used the validated paper version of the Self-BAT to create an online version.

The Self-BAT uses simpler language and is a shorter questionnaire that can be completed from a person's own home. Patients are able to bring the results of their questionnaire, which serves as a comprehensive bleeding history, with them to their medical appointment.

The study investigators noted, "We have recently shown that individuals referred to [hematology] for an abnormal online Self-BAT were more likely to require interventions compared [with] those referred for other reasons." Such a statement implies that the online Self-BAT has the ability to recognize those who might benefit from treatment with improved accuracy compared with standard referrals.

In the current study, the authors validated use of the online Self-BAT as a screening tool for bleeding disorders among a population of patients who were new referrals to the hematology clinic. Study participants were grouped according to diagnosis: a bleeding disorder or no bleeding disorder. A bleeding disorder of unknown cause was considered a bleeding disorder diagnosis based on an abnormal bleeding score.

Between 2016 and 2024, 63 female patients were recruited. One male patient completed the study but was excluded from analysis because of a normal Self-BAT. Overall, 42 of the 63 participants were diagnosed with a bleeding disorder and 21 were not. In the group diagnosed with a bleeding disorder, mean self-BAT and ISTH-BAT scores were statistically significantly higher than the scores among those without a bleeding disorder (P <.001).

Findings from intraclass correlation coefficient showed a significant positive association (0.738; 95% CI, 0.601-0.833; P <.001) between total scores on the Self-BAT and ISTH-BAT. Similar to findings on the paper Self-BAT version, an abnormal Self-BAT (ie, a score of 6 or higher among females) had a sensitivity of 95.2% and a negative predictive value of 77.8% for identifying a bleeding disorder using the expert assessment outcome as the gold standard (ie, diagnosed or not diagnosed). The ISTH-BAT had an equal sensitivity of 95.2% and an negative predictive value of 87.5%.

"This study demonstrates that the online Self-BAT can identify bleeding disorders with an equal sensitivity in comparison to the expert-administered ISTH-BAT without patients needing to leave their home, and without time and resource expenditure during medical appointments," the authors highlighted.

Because of the "small sample size, there was a lack of male representation in this population," they noted. Although "most patients referred to the clinic are female, this limits the study's generalizability to males. Bleeding disorders present differently in males, and as such, their symptoms are interpreted differently in the Self-BAT with a lower threshold for abnormal," the authors concluded.

This article originally appeared on: https://www.rarediseaseadvisor.com/news/self-bat-screen-carriers-bleeding-disorders-hemophilia/

The sensitivity of the online Self-BAT was 95.2%, which was the same as that of both the paper Self-BAT and the ISTH-BAT.

Upcoming Events

30 Jan-1 Feb 2026
Auckland
Teen and youth camp

19-22 April 2026
Kuala Lumpur
WFH 2026 World Congress

2-4 October 2026
Auckland
Women's wellness weekend

21-24 January 2027
Christchurch
National family camp

CONTACTS

National office

44 Victoria Street
Alicetown
Lower Hutt 5010

info@haemophilia.org.nz
www.haemophilia.org.nz
Free phone: 0508 FACTOR (322867)

Chair
Hemirau Waretini
hemi@haemophilia.org.nz

Chief Executive
Deon York
ceo@haemophilia.org.nz

Communications Manager
Phil Constable
comms@haemophilia.org.nz

Administrator
Leanne Pearce
03 371 7477
admin@haemophilia.org.nz

Southern Outreach
Vic Turner
03 371 7485
vic@haemophilia.org.nz

Central Outreach
Lynne Campbell
04 382 8442
lynne@haemophilia.org.nz

Midland Outreach
Loren Silva
07 856 4442
loren@haemophilia.org.nz

Northern Outreach
Darian Smith
09 845 4658
darian@haemophilia.org.nz

4 WAYS you can support Haemophilia NZ

Our work on behalf of all kiwis with a bleeding disorder is only made possible with your generous support.

1. Your **membership** helps us do more

You can join or renew your membership at **haemophilia.org.nz**

2. Leave a **lasting legacy**

Honour the memory of a loved one, or recognise the special bond you have with HNZ by leaving a bequest on your will.

Find out about leaving a bequest at **haemophilia.org.nz**

3. **Make a donation**

We are always very grateful to receive your kind donations at any time. You will find a “Donate Now” button on our home page at **haemophilia.org.nz**

4. Read Bloodline online at any time

Reading Bloodline online saves trees and us money!

You will find the latest issue at **haemophilia.org.nz**

Haemophilia New Zealand