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The H Word

Tēnā koutou katoa,

It is with great pleasure that I introduce myself as the new Chair of Haemophilia New Zealand (HNZ). Like many of you, my journey with HNZ began as a child, attending family camps that shaped lifelong friendships and connections within the bleeding disorders community. It has been a great pleasure to reconnect with many of you and meet new families at recent regional camps in the Central and Southern regions.

In my teenage years, I moved from camp participant to camp leader, learning from older leaders, outreach workers, and support staff. These experiences led me to join the Northern Regional Committee, the HNZ Youth Committee, and Piritoto Committee. Working alongside the incredible staff, treaters, and volunteers in our community has been an enriching and humbling journey.

HNZ has a long-standing history and a global reputation for delivering high-quality education, advocacy, and support programs while championing the best treatment and care. I am committed to building on this legacy as I support the board and our dedicated staff in continuing this vital work.

My involvement at the governance level began in 2012 when I joined the National Council. In 2014, I stepped into the role of Treasurer. Since then, HNZ's balance sheet has grown from under $800,000 to over $2.7m, largely due to generous public donations. This financial foundation allowed us to achieve a significant milestone: the purchase of a property in late 2022 that now serves as a home for bleeding disorders in New Zealand. This property not only provides rental income but also offers a workspace for our Wellington-based team.

While we celebrate these successes, we must remain focused on sustaining our financial base amid challenging economic conditions and limited grant opportunities. This stability ensures HNZ can continue delivering critical services to our community across Aotearoa.

Over the years, advancements in treatment have transformed the lives of many in our community. Early in my volunteer journey, treatment was largely limited to conventional factor replacement therapy. Thanks to extensive advocacy, we now have access to extended half-life treatments and innovative therapies, significantly improving quality of life for many members notably the funding of emicizumab (Hemlibra) in October 2023. HNZ will continue to advocate for the best in treatment and care for all people with bleeding disorders.

The improvement in treatments for many of those with the most severe conditions gives us more capacity to turn our attention to those who have not always been as visible. One such area is the experience of women and girls with bleeding disorders, an issue gaining international attention under the 2025 World Haemophilia Day theme, "Access for All: Women and Girls Bleed Too." HNZ, led by Lauren Phillips and Tineke Moate, has introduced a pilot program providing period products to people who menstruate and have a bleeding disorder.

International engagement continues to play a vital role in our work. Through my participation in programs such as SURO, AFFIRM, and the World Federation of Hemophilia twinning initiative with Nepal, I've seen first-hand the importance of staying connected to the global bleeding disorders community. These connections ensure we remain informed about best practices and emerging treatments, which we bring back to New Zealand for the benefit of our members.

The COVID-19 pandemic presented challenges to our ability to deliver programmes, but recent national events such as the New Families Camp, Teen and Youth Camp, and Women's Weekend have shown an enthusiastic resurgence in participation, as well as the record numbers at camps and event organised and run by regional committees. Looking ahead, we have a full calendar, including the New Families Camp in February and an Adult Wellness Weekend later in the year. Our regional branches (Northern, Central, Midland, Southern, Piritoto, and Youth) have also received additional funding to support their activities in FY24/25. If you're interested in helping organise or support regional events, I encourage you to contact your outreach worker.

In the past year, HNZ also underwent a governance review to strengthen our structure and better align with our mission of providing excellence in education, care, advocacy, and support. Key changes include longer board terms to foster continuity, build expertise, and maintain strategic focus.

As we are preparing to review our strategic plan in the new year, we'll draw on insights from the recent Member Survey to ensure we're addressing the needs of our diverse community. Together, we'll identify the programs and services that will have the most meaningful impact.

It is an honour to take on this role, and I am inspired by the dedication of the board, staff, and volunteers who make HNZ what it is today. Over the next four years, I look forward to working with you all to continue making a positive difference in the lives of those affected by bleeding disorders.

Mauri ora,

Hemirau Waretini

Chair, Haemophilia New Zealand

WFH 2024 World Congress

The World Federation of Hemophilia 2024 World Congress took place in Spain from 21 - 24 April. HNZ sent five members and staff to attend this fantastic event. This was an important opportunity for our people to learn and share on the world stage. The HNZ group attended as many sessions as they could and have been hard at work since putting together their reports. Three attendees reported in the previous issue, the remainder report here.

Karl Archibald: HNZ Treasurer

Global NMO Training 2024

The World Federation of Hemophilia (WFH) hosted its 2024 Global NMO Training on April 19-20. This two-day event aimed to equip National Member Organizations (NMOs) with the knowledge, tools, and networks needed to improve care for people with bleeding disorders (PWBD).

Through a mix of presentations, interactive workshops, and informal networking opportunities, the training enabled shared learning among participants from around the world.

The agenda covered key themes such as good governance, outreach and diagnosis strategies, youth engagement, and the application of project frameworks.

Each session brought a unique perspective, reflecting the expertise and diversity of the presenters and participants.

Day One

Opening Plenary: Addressing the Global Diagnosis Gap

Salome Mekhuzla, WFH Director of Global Development, opened the training by highlighting the global challenges in diagnosing PWBD. According to the WFH 2022 Annual Global Survey, only 34% of people with haemophilia have been identified. This statistic underscored the urgency of scaling outreach and diagnostic efforts.

Salome outlined several WFH initiatives, including:

- **Pilot Outreach Projects**: Workshops in Ivory Coast and India introduced training programs to enhance diagnostic capabilities.
- **Laboratory Training Programs**: Regional and national workshops, coupled with healthcare development programs, aimed to standardize diagnosis practices.
- **International External Quality Assessment Scheme (IEQAS)**: Over 20 years, this initiative has enrolled 145–150 laboratories annually to improve diagnostic accuracy.

Dr. Sergio Robledo Riaga from the Liga Colombiana de Hemofílicos presented findings from the HEMDATA program

- **New diagnosis**: It identified 426 new women with bleeding disorders. This project emphasized the need to educate healthcare providers about recognizing symptoms in women, who are often overlooked in diagnosis efforts.
- **Spain's Collaborative Model**: Laura Quintas Lorenzo from the Federación Española de Hemofilia (FEDHEMO) described partnerships between gynaecologists and haematologists to address gender-sensitive issues in VWD care. FEDHEMO's webinars and roundtables engaged thousands, with recordings achieving over 50,000 views.

Roisin Burbridge from the Irish Haemophilia Society discussed Ireland's success in raising awareness of VWD.

- **Campaigns** leveraging social media, radio, and newspaper outreach achieved a 9% increase in adult diagnoses since 2020.
- **Metrics** showed social media posts reached 20,400 people, and paid advertisements brought consistent traffic to educational resources.

These cases demonstrated the importance of tailoring awareness campaigns to local contexts and leveraging partnerships to maximize impact.

These efforts aim to close the diagnosis gap and ensure equitable access to care worldwide.

Good Governance in Hemophilia Organizations

Carlos Safadi Márquez, WFH Vice President for NMO, presented on the importance of good governance in haemophilia organizations. They defined governance as the framework for decision-making and accountability, emphasizing its role in ensuring the sustainability of NMOs.

Key Topics:

- **Participation and Equity**: Carlos stressed the importance of inclusive decision-making that involves all stakeholders, from board members to community representatives.
- **Transparency and Accountability**: Underscored the value of clear policies and financial management practices to build trust with donors and beneficiaries.
- **Succession Planning**: Carlos highlighted the need for proactive leadership development to ensure continuity.

This was the precursor to the practical sessions later explored how NMOs could implement governance principles in areas like risk management, external relations, and strategic planning.

Youth Inclusion in Governance

Amierul Hakimie Luqman of the Hemophilia Society of Malaysia led a session on incorporating youth perspectives into organizational governance. He described how the society established a Youth Committee, with 4 out of 9 board members being under 30.

Session Insights:

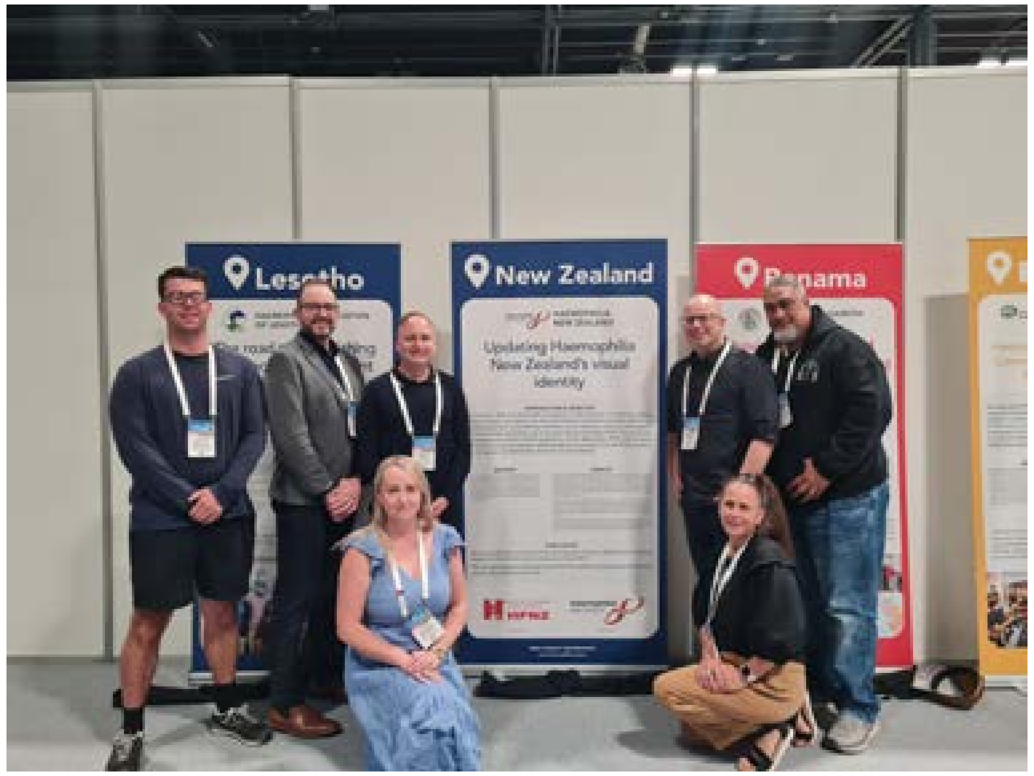
- **Engaging Activities**: Youth retreats and camps were instrumental in building confidence and leadership skills among young members.
- **Challenges and Solutions**: Amierul discussed the difficulties of integrating youth perspectives into decision-making but emphasized the long-term benefits for organizational growth.

Governance in Action: Haemophilia New Zealand

I presented HNZ's initiatives and achievements over the last 24 months and steps HNZ have taken to modernize governance and the organisation.

Key achievements:

- **Refreshed** policies and constitution, with a diverse board of eight volunteer directors, and the recently simplified language, and updated board terms.



- **Period Equity Pilot Program** to provide free period products and granting resources for education, fitness, and other needs.
- **HNZ's Updated Visual Identity**, encompassing principles of Whanaungatanga, Mātauranga and Kaitiakitanga.
- **Engagement** I discussed the value of having the right skills and people in the right roles.
- **Twinning** highlighted HNZ's partnership with the Fijian Haemophilia Foundation, which included diagnostic and governance support.
- **Home for Haemophila New Zealand**: the long team process to purchase a property and embarking on a social

enterprise along the way.

Day Two

We participated in practical workshops involving budgeting, project development, identifying stakeholders and MEAL methodologies. These were facilitated by WFH Staff and volunteers.

Practical Tips for Budgeting and Reporting

This workshop focused on budgeting and financial reporting for NMOs. The aim was to provide tools and tips to help manage funding effectively and show funders the real impact of the work in the bleeding disorder community.

Key Topics Discussed

1. Budgets are more than numbers; they tell a story. They:

- Outline what we plan to do with funding.
- Show how we will do it.
- Highlight the impact it will create in the community.

2. Creating a Strong Budget

- Identify all the costs involved in your activity.
- Obtain at least two comparable quotes for accuracy.
- Break expenses down into specific items.

3. Tracking Spending

- Record expenses as they occur, keeping receipts and invoices organized.
- Compare actual costs to your budget regularly to spot and explain any differences.

4. Reporting to Funders

- Funders expect transparency and detail.
- Share how their money was spent and the outcomes achieved.
- Use round numbers and explain major assumptions (e.g. exchange rates or inflation).

Interactive Activity: Training for Health Workers

Irene further discussed that transparency creates credibility and encourages funders to provide future grants. Planning helps prepare for future projects and fosters and by strengthening internal processes and financial management it improves accountability.

We practiced creating a budget for training to raise awareness about bleeding disorders. Key steps included:

- Listing expenses
- Providing cost breakdowns
- Using a budget template to organize and present the data clearly.

Takeaway Message

A good budget is the foundation of a successful project. It helps you plan, stay on track, and show funders the value of their support. By managing funds effectively, NMOs can create a bigger impact and build trust for future collaborations.

Project Proposal Development

The workshop focused on applying project management principles to develop effective project proposals. We learned to define project scope, engage stakeholders, and align activities with strategic goals. Tools such as SMART objectives and logic models were used to enhance project planning, execution, and monitoring.

Key Topics Covered:

Initiating and Planning the Project

During the initiation phase, we identified the problem statement, target population, and stakeholder needs. A Strength Weakness Opportunity Threat (SWOT) analysis was recommended to evaluate internal and external factors that impact project feasibility and alignment with organizational strategy.

For planning, we learned to define the project scope through SMART objectives. These ensure the project is Specific, Measurable, Achievable, Relevant, and Time-bound, creating a clear roadmap for deliverables and constraints.

Using the Logic Model as a Planning Tool

The logic model was introduced as a structured framework for decomposing projects into manageable components:

- **Inputs (Resources)**: Financial, human, and technological assets aligned with the resource management plan.
- **Activities (Tasks)**: Defined actions linked to deliverables, forming the basis of the project schedule.
- **Outputs (Deliverables)**: Measurable products, such as the number of trained individuals or educational materials distributed.
- **Outcomes (Short/Medium-Term Results)**: Indicators of success, like increased awareness or engagement.
- **Impact (Long-Term Goal)**: Reflecting the strategic benefits of the project, such as improved healthcare access.

Practical Exercises

Participants developed SMART objectives and logic models using a case study. These hands-on activities provided experience in translating project goals into actionable plans while ensuring alignment with stakeholder expectations and scope.

Key Takeaways

- **Stakeholder Engagement**: Engage diverse stakeholders to ensure alignment with needs and priorities.
- **Scope Management**: Use SMART objectives to define and track deliverables.
- **Resource Management**: Allocate and monitor inputs for effective execution.
- **Monitoring and Evaluation**: Use logic models to connect activities to outcomes and validate project success.

By combining clear objectives with structured planning, projects can deliver measurable outcomes while meeting stakeholder and organizational expectations.

Monitoring, Evaluation, Accountability, and Learning (MEAL)

Key Topics Covered:

What is MEAL?

MEAL provides a structured approach to measure, track, and evaluate projects:

- **Monitoring**: Tracks activities and outputs to ensure alignment with plans and identify improvements.
- **Evaluation**: Assesses outcomes and impact, ensuring project sustainability and effectiveness.
- **Accountability**: Promotes transparency and stakeholder participation, building trust and engagement.
- **Learning**: Encourages reflection and adaptation to improve future project design and implementation.

MEAL builds on traditional Monitoring & Evaluation (M&E) by incorporating accountability and learning, emphasizing stakeholder involvement and continuous improvement.

Data Collection Tools and Methods

We explored tools to gather insights from stakeholders and support decision-making:

- **Surveys**: Collect quantitative and qualitative data.
- **Interviews and Focus Groups**: Provide in-depth perspectives from stakeholders.
- **Document Reviews**: Analyze reports and policies for progress tracking.
- **Observations**: Capture real-time insights on activities.
- **Case Studies and Social Media Analytics**: Offer success stories and track online engagement.

Each tool was discussed in the context of practicality, project goals, and target audiences.

The Budget, Project MEAL.

The workshops linked back with the earlier examples in the plenary sessions. Together, these elements create a framework where participants were able to take away budgeting, planning, execution, and evaluation tools with tangible examples to build momentum for NMO success.

Final thoughts

Beyond the formal sessions, the training offered opportunities to reconnect with old friends and network. Whether during the breaks or shared moments like being stuck in a lift. NMO Training was an example of the benefits of collaboration and shared learning. It provided participants with practical tools, new perspectives, and strengthened networks, all aimed at improving care for PWBD.

Connor McCone: HNZ Board youth delegate

Attending the 2024 World Federation of Haemophilia (WFH) World Congress in Madrid was an amazing experience and a significant milestone in my journey within the bleeding disorder community. Hosted at IFEMA Madrid, the Congress brought together experts, advocates, and people with bleeding disorders (PWBD) from around the world, united in their commitment to improving lives through education, advocacy, and innovation.

Personal Reflections and Key Highlights

This was my first WFH Congress and a long-awaited bucket list item. It was inspiring to meet and learn from brilliant individuals such as doctors, researchers, nurses, policymakers, and PWBD who dedicate a good chunk of their lives to addressing the challenges of bleeding disorders. Representing New Zealand at the General Assembly alongside Tineke Moate was particularly meaningful. It gave me a sense of pride in our country's contributions to the global haemophilia community.

A standout moment was witnessing Deon receiving an award, a testament to the incredible talent and dedication within our community. Additionally, I was incredibly proud of the high regard in which other New Zealand representatives, including Karl Archibald, Lauren Phillips, Hemi Waterini, and Catriona Gordon, are held internationally. HNZs efforts to advocate for bleeding disorders are widely respected and it was awesome to see this recognised!

Key Congress Themes

The Congress addressed a wide range of topics critical to the bleeding disorder community, organised into various tracks:

- **Medical Advances**: Innovative discussions covered gene therapy, novel treatments, and the potential for curative therapies. Learning about the advancements in gene therapy was particularly inspiring, offering hope for a future with fewer medical challenges for PWBD.
- **Women and Girls with Bleeding Disorders (WGBD)**: This track highlighted the unique challenges faced by women and girls, who historically received less recognition in the bleeding disorder narrative. The discussions emphasised the importance of equitable access to diagnosis, treatment, and psychosocial support.
- **Psychosocial Support:** These sessions underscored the importance of addressing mental health and emotional wellbeing in managing bleeding disorders. They highlighted strategies for building resilient support systems and fostering community engagement.
- **Nursing and Musculoskeletal Care**: The Congress included specialised sessions for nurses and explored innovative approaches to managing musculoskeletal complications, a common issue for PWBD.
- **Laboratory Sciences and Dental Care**: Advancements in diagnostic technologies and oral health strategies were also focal points, emphasising a multidisciplinary approach to patient care.

A Future Focus on Inclusivity

One of the most impactful themes was the Congress's focus on inclusivity and the future of care. There is a growing commitment to supporting women and girls with bleeding disorders, who have often been overlooked. Similarly, the Congress emphasised the need for enhanced psychosocial support to ensure holistic care for all individuals regardless of gender.

Networking and Global Collaboration

A highlight of the Congress was the opportunity to network with peers from different regions. Many participants recognised the New Zealand lanyard and shared their respect for our community's contributions.

The General Assembly, which welcomed new member organisations and expanded the WFH network to 152 NMOs, showcased the growing global commitment to advancing care.

In my opinion the 2024 WFH World Congress was more than just a professional gathering; it was a celebration of resilience, innovation, and hope within the bleeding disorder community. It was inspiring to see the collective efforts to push boundaries, advocate for equitable care, and ensure a brighter future for all individuals living with bleeding disorders.

For me, this Congress reinforced the importance of continued involvement and advocacy. It was a privilege to be part of such a dynamic event and to witness firsthand the profound impact of global collaboration. I am excited to bring back the knowledge and insights gained from this experience to further contribute to New Zealand's efforts in the bleeding disorder community.

A new Chief Executive for Haemophilia New Zealand

Our September Pānui shared that Deon York was appointed Chief Executive of HNZ. While Deon is no stranger to the community by any measure, Darian Smith, Northern Outreach, sat down with Deon to find out more about him, the year that has been, and how he feels about his appointment.

You are certainly well known to most of our members, but to some you might be a complete mystery. Tell us a bit about yourself and maybe something that the members don't know about you?

Well, it is certainly no secret that I have severe haemophilia A, and as a child of the eighties my parents had to live through the HIV and HCV era which could be a whole separate story for another time. My father was from Liverpool, and my mother was born in Gisborne. I grew up in Wellington and that's where I currently live with my partner. I am an only child and it's not true what they say about only children! (Which is something an only child would say.)

I do have three half-sisters (one who fairly recently passed away) and many nieces and nephews spread out around the country and beyond. Sadly, both my parents have also passed away. They were my greatest support growing up and taught me how to advocate for myself and others. I think people already know that every job I have ever had has been connected to our health system in some way. But what's something that people don't know about me? Well, some people might not know that I used to play the piano accordion (I even busted it out at one of the camps many years ago).

Up until recently you were the chair of Haemophilia New Zealand and have been in voluntary roles for the organisation for around 25 years at last count. What has kept you motivated for so long?

The members of this organisation are inspirational. We all live in the same country, yet health outcomes vary – even within our membership. That is unacceptable and unnecessary. There are many reasons for this which I won't go into here but seeing where we are and where we can be is a constant motivation.

Over these many years, I have seen an increase in the breadth and depth of events, as well as educational opportunities for our members. I have noted the continuous improvement of care and treatment for people with haemophilia and related inherited bleeding disorders. The recognition of HNZ as a trusted advisor has improved year on year. We should all be proud of the steady growth of HNZ.

Of course, the work of a chair is never done. It was also important I saw through two major milestones for the organisation before handing over. In the last quarter of 2023, emicizumab (Hemlibra) was funded for all people with moderate and severe haemophilia A, representing the biggest shift in therapy for years. To me, the advocacy in the background, the patience, and the perseverance have all paid off. We will continue to follow the trends for the haemophilia B treatment pipeline, von Willebrand's, and rare factor deficiencies.

The second major milestone to see through was the 'home for HNZ' which has been often quoted to members and has been a dream for so long. While a formal opening has been planned, the office is now open, functioning, and ready to welcome members. The board had its first meeting there on 23 November and we hosted the AGM as well. I know this has been the vision of so many staff and volunteers of the organisation over the years, and it is exciting and rewarding to see it come to fruition after so long. Reaching this long-held aspiration has been a truly rewarding experience.

What is your proudest achievement as chair?

Seeing through the two major milestones of Hemlibra being funded and the property finally opening mean so much to me because of what they mean for our members. For a small country, having access to this therapy and having a permanent space to call ours is truly remarkable. Of course, these milestones give a sense of achievement and there are so many people to thank who contributed to making this vision a reality. My true sense of pride comes from our members. We all face different realities but share the experience of navigating life with a bleeding disorder. Members everywhere are living remarkable lives. Even the seemingly ordinary is extraordinary. I know there are people dragging themselves to work with a sore ankle, having periods that last weeks and getting on with it anyway, going to school with sore joints and not complaining about it, and still living life and making it one way or another. How can we all not be inspired by this?

You are the fourth chief executive for HNZ. How do you feel, ten weeks in, about taking on this role?

I am honoured to take up this position. There is something special about working for an organisation you have a deep affinity with. There is plenty of work to do, and the people continue to inspire me to work every day to ensure the organisation is strong and delivering for its members. We have so much to be proud of as an organisation, and I know we can do more.

When I began as chair (we won't go back further!), HNZ was an organisation with accumulated assets of $650,000. In our annual report which reports up to 30 June 2024 this year, we are now the custodians of assets and accumulated funds of more than $2.7 million. Every dollar is testament to stellar fundraising across the country, volunteer contribution, the grant support we have received over the years, the generosity of the public, and prudent investment. Our role as a not-for-profit is to spend funds for our charitable purpose, although this must be tempered with sound financial management. It is also important to note that many funds have specific tagged purposes or are accumulated assets and therefore not 'liquid'. No one can predict future financial circumstances, but we can always plan for them.

Any former chief executive of this organisation will tell you that while fundraising is not the only purpose of the position, and we do have Kiwifirst as our principal fundraiser, raising additional funds remains a constant challenge, particularly in this environment. So, while I am already focused on many projects, it is the raising of funds that I am very thoughtful about, tempered with financial management of the organisation.

What advice do you have for the incoming board and chair?

I think what I had to say in the annual report this year sums it up. I can say with confidence that the organisation is in excellent shape and well set up for the future. My advice to any future board, staff, or volunteer is:

- Stay true to our mission: Care, education, advocacy, and support for all people with haemophilia and related bleeding disorders. The emphasis is on all – in other words, no one should be left behind.
- Constantly follow trends in the latest therapies and treatment: No one, not even the system itself, will take a greater interest in the potential new therapies on offer than you.
- Continue to expand our horizons: Consider how to bring talent in from both our members and beyond. Remain committed to continuous improvement for both the organisation and our members.
- Always remember our strength: Our people are our greatest asset and HNZ exists to serve our people. Our connections run deep, and our shared history binds us together. This is our strength.

Now you are the chief executive, what's your vision for HNZ and how do you plan to achieve that?

All my predecessors in this role kept to the vision of improving the lives of people impacted by bleeding disorders, and I do think the organisation is currently in excellent shape. People's lives have changed significantly over the past few years and so I think it is time to set a revised vision and strategic plan for this organisation, and I look forward to working with the board to devise this and then put it into action.

My vision is high quality care for our members everywhere, regardless of where they live. I understand the challenges the current system faces and I equally understand the challenges our members face. Having a bleeding disorder is not a choice, and where you go to get treatment is not a choice, either. No matter where you are born or who you are, you should have access to equitable treatment and care. We do quite well in New Zealand, but is 'quite well' what we ultimately aspire to?

I think we need to keep the momentum going with our activities for members and better understand and measure the impact we are having, hold our position as a trusted advisor, and continue to manage our finances carefully. We need to remain educated and informed about haemophilia and all related inherited bleeding disorders. I will continue to be driven by these goals.

I also look forward to supporting Lauren and Tineke as the continue with the period project for our women and girls affected by bleeding disorders.

By the time of Bloodline publication, a ‘twinning’ visit will have taken place in Fiji which will now include the new chair Hemi and Dr Julia Phillips. This is the third year of a four-year twinning programme with Fiji is taking place focusing on building the capacity of the Fiji Haemophilia Foundation alongside diagnosis and treatment. All the ‘ingredients’ are there, and we are so close to making a real impact. Along with Dr Phillips, we have been focusing on how to build lab capacity to formally diagnose people in Fiji so appropriate humanitarian aid can be given. Pacific nations are not far from New Zealand. While we are geographically close, the gap between treatment and care is much vaster than the oceans that separate us.

HNZ has also been approved for a ‘youth twinning’ following a successful assessment visit to Pakistan joined by Connor McCone as the youth delegate to our board. Connor will be leading this youth twinning in 2025. I raise these alongside our local activities because we should remind ourselves of our commitments to being a responsible member of the global bleeding disorders community and help where we can. No charitable funds raised in New Zealand are used for this purpose, so being responsible globally has a minimal financial impact. When you know the realities of no treatment, how can we not continue to support our global friends and partners.

The year is almost over. How has it been for you?

I end the year with gratitude. Thank you to the members and staff wholeheartedly for their support this year, and over many years. The memories I am surrounded by in this new office are inspiring, and I hope that many of our members can enjoy this space as I am. This financial year has already been a busy one, starting with a women’s wellness weekend and regions planning and delivering large events. We have a family camp coming up in February 2025 and an official office opening for HNZ in May among many other things.

Whatever you are all doing over the break, I do hope you get the opportunity for some relaxation, and I can’t wait to launch into 2025!

Receiving the World Federation of Hemophilia’s International Frank Schnabel Award

Earlier this year, Deon York received the World Federation of Hemophilia’s (WFH) prestigious International Frank Schnabel Volunteer of the Year award at the World Congress of WFH in Madrid. Darian Smith was there and witnessed the moment first-hand. Darian continues his interview with Deon and asks him about the special moment.

I was there when you received the award from the World Federation of Hemophilia (WFH) in Madrid while watching from the audience. I did a bit of research on the award and learnt that it was established in 2004, named in honour of WFH founder Frank Schnabel. It is given to an individual with haemophilia, an inherited bleeding disorder, or to a family member, who has contributed significantly to furthering the mission and goals of the WFH, both in your country of origin and globally. Tell us about the experience for you.

For one, it was a complete surprise! It was a well concealed secret. As I heard my name being called at the opening ceremony, I experienced one of those moments when you think it is meant to be someone else. I was in total shock and disbelief, and to top it all off I was completely speechless! If this was not moving enough, as I stood on the stage to receive the award, attendees from across the globe witnessed a haka in the distance performed by Tineke, John, and Lauren. It was a truly moving experience for me and a new experience for the audience outside of NZ.

There are many people all over the world volunteering for our communities. Why do you think you received the award?

Indeed, this was what shocked me. I have worked with many remarkable leaders around the world who have achieved incredible feats for our communities, and learnt a lot from them. I think I am willing to put in the long hours, pay attention to the details, and look at how to solve problems together. More importantly, simply applying a cookie cutter approach to different counties with specific cultural and social norms will not yield any result. Bleeding disorders are all over the world and how they impact people can be influenced by not only the resources a country has, but also the context. You must be willing to adapt, grow, have your perspective challenged, be outside your comfort zone, and respect the space you are walking into.

What has working globally taught you and how does it benefit people here?

I have seen the reality of living with a bleeding disorder with limited to no treatment around the world. More than 70% of the world remains in this position. The people I have met have built so much with so few resources and how they have managed to do this drives me to think about how we can do more and go further with what we have in New Zealand. We may be very fortunate to have treatment and care here, but there is also much we can learn from others. The way we collect data and use it to deliver results, and the way we engage with our members, for instance, can be improved and we can learn from our global community how to do this and strive to be better.

The WFH gave you this award. The WFH is a non-profit organisation dedicated to improving and sustaining care for people with inherited bleeding disorders around the world. What is your connection to WFH?

I was the first New Zealander to be elected to the board of the WFH in 2010 and served two elected and one co-opted term until my last term concluded in 2020. In my second elected term I received a majority of support from other countries to continue. Over the years I have chaired and worked with many WFH committees, initially in youth development and fundraising, then branching out to research, congress organisation, and many other areas of the organisation. I continue to maintain a strong connection with the WFH. The most recent examples are the twinning programmes with both Fiji and Pakistan.

For those of us present in Madrid, we really could see how significant receiving this award was. It certainly put New Zealand front and centre and on the global stage. Congratulations again!

WFH Global Forum

Hemi Waretini: HNZ Chair

Advancements in Haemophilia Care: Insights from the WFH Global Forum

Through the AFFIRM program, supported by the Global Blood Disorder Foundation, I attended the World Federation of Hemophilia (WFH) Global Forum in Montreal, Canada. The event gathered experts, advocates, and stakeholders to discuss developments and challenges in bleeding disorders care.

With new therapies emerging, the forum provided a platform to review advancements, assess their potential, and address persistent barriers. Topics ranged from innovative treatments to the challenges faced by individuals living with bleeding disorders.

Addressing Challenges in Haemophilia Treatment

A key theme of the forum was the limitations of existing therapies and the need for new approaches. Discussions focused on:

- **Global Plasma Supply**: A critical issue, particularly in low-resource settings dependent on plasma-derived treatments.
- **Platelet Disorder Therapies**: Addressing diagnostic and management challenges.
- **Care for Women and Girls**: Highlighting gaps in equitable treatment and overlooked needs.

Advances in haemophilia treatments were explored, including replacement therapies, non-replacement approaches, and gene therapy. Emerging strategies like rebalancing agents and gene therapy were examined for their benefits and limitations.

Equity and Access in Care

The forum emphasised equitable access to treatments, with discussions on affordability and availability. Experts shared strategies to overcome market access barriers and economic challenges, particularly for gene therapies targeting haemophilia A and B.

Speakers from diverse regions offered global perspectives on addressing inequities, underlining the importance of collaboration in creating sustainable solutions to expand access to advanced therapies.

Rebalancing Agents: A Promising Innovation

Rebalancing agents were highlighted as a potentially transformative approach to bleeding disorders. These therapies are still being developed and studied, so it is uncertain when or if they will come on the market.

Instead of directly replacing the missing clotting factor (like factor VIII or IX), they target natural anticoagulants (the body's clot-preventing mechanisms). By reducing the activity of these anticoagulants, these drugs "rebalance" the clotting system to work more effectively, even without the missing factor. Rebalancing agents have the potential to treat multiple bleeding disorders, not just haemophilia. This is because they target broader mechanisms in the blood clotting process, rather than relying on the specific clotting factors that are deficient in each condition.

Types of Rebalancing Agents:

- **Antithrombin Suppressors** (e.g., Fitusiran)
  - Reduce antithrombin to improve clot formation.
  - Benefits: Effective for haemophilia A and B, including patients with inhibitors, with convenient monthly dosing.
- **Tissue Factor Pathway Inhibitor (TFPI) Blockers** (e.g., Concizumab)
  - Block TFPI to strengthen clot initiation.
  - Benefits: Suitable for multiple bleeding disorders, including inhibitor cases, with subcutaneous delivery.
- **Other Targets**
  - Therapies exploring inhibition of Activated Protein C (APC) to enhance clotting further.

These agents offer new hope for patients with limited treatment options, expanding therapeutic possibilities.

Looking Forward

The WFH Global Forum underscored the importance of bridging gaps in accessibility, affordability, and education to ensure advancements in care reach all patients. From rebalancing agents to equitable healthcare strategies, the forum highlighted the global community's commitment to improving outcomes for people with bleeding disorders.

The Allan Coster educational endowment trust

The Allan Coster Educational Endowment Trust (ACEET) is a charitable trust that offers grants to HNZ members with haemophilia and other rare bleeding disorders to help them access academic or employment-related study. Over the years, ACEET has helped many people to change their lives, providing them with the financial support for their education that they may not otherwise have been able to access.

The ACEET grants are not just for university study. The ACEET trustees will consider applications for primary, secondary, undergraduate, postgraduate, trades, or vocational training. That means, if you have a bleeding disorder, a dream, and a course that will help you to get where you want to be, then an ACEET grant could be what you need.

ACEET is administered by three trustees, who make decisions about how the capital is managed and how grants are distributed. Recently there has been a slow changing of the guard, as long-serving trustees step back, and new ones are appointed.

HNZ patron, Dr Elizabeth Berry, and former HNZ President, Mike Carnahan, have given many years of sterling service to ACEET. Last year they announced their intention to retire, and the search began for two new trustees to work alongside Dr Paul Ockleford managing the trust. We are very grateful to Elizabeth and Mike for all the time and effort they've put in to supporting the advancement of HNZ members.

While the management structure of ACEET changed, they paused offering grants. Now two new trustees have been appointed, and they're ready for grants to be offered once more. We are very pleased to welcome former HNZ outreach manager Colleen McKay and Professor Julie Park, who is a social, cultural, and medical anthropologist and bleeding disorder researcher. We know that the new trustee team will make good decisions about future applications.

Previous recipients have used their grant to complete courses from reading assistance, to business management, to graphic design. Here's what some recent recipients had to say about their ACEET experiences:

"Studying law meant expensive books, expensive course fees and, being in Wellington, expensive coffees! ACCET couldn't help me with the last one but it did assist me with course materials - which when you're paying $160 for a 2 cm thick book is very handy! I was successful in obtaining a grant through the Trust to assist me but perhaps more important than the financial assistance, it felt like a vote of confidence from the trustees. They believed in the work that I was doing and wanted to support that."

"As my health conditions already make university on its own difficult for me to manage, I am understandably unable to take on any part time work while in school, so receiving the ACEET grant was a major weight off of my shoulders, as it made me able to afford the materials and supplies I need for my (fashion design) course."

If it is going to improve your prospects, then it's worth getting an application in. Grant applications are considered three times a year. To check the next application deadline, or apply for a grant, just contact your outreach worker.

- **Northern**: Darian Smith - 027 512 1114 or darian@haemophilia.org.nz
- **Midland**: Loren Silva - 021 762 121 or loren@haemophilia.org.nz
- **Central**: Lynne Campbell - 027 273 3443 or lynne@haemophilia.org.nz
- **Southern**: Vic Turner - 021 656 804 or vic@haemophilia.org.nz

HNZ women's wellness weekend 2024

On 26 - 28 July women from across the country gathered in Taupō for the 2024 HNZ women's weekend. Traditionally haemophilia and bleeding disorder education has been geared towards men. This educational weekend 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.

The women rolled into Taupō on Friday and began the weekend with a fantastic introductory session, run by Iona Kahu. This was the perfect way to get the weekend going, and gave the attendees the opportunity to find out more about each other and to settle in. The women really enjoyed this session and felt that having at icebreaker made a huge difference to how the weekend got underway.

With the ice broken it was time for dinner and our special after-dinner speaker. We were lucky enough to have HNZ board member Lauren Nyhan Zoom in to talk about bleeding disorders and intimacy. Lauren was a guest speaker at the recent World Federation of Hemophilia World Congress, where she addressed the same topic. It was so valuable to have the opportunity to discuss what can be a challenging topic in such a safe and supportive environment.

The result of these, quite personal, opening day activities was that the women very quickly became comfortable with one another. This allowed for some excellent engagement and participation as the weekend went on. The personal stories shared were inspiring and led to a safe space for others to share. It was lovely to see women connecting and feeling heard across generations.

Day two saw the start of our education programme. A big part of these weekends is about learning skills and information to help women deal with their bleeding disorders. Women with bleeding disorders often spend a lot of their time and emotional energy supporting other members of their whānau, who almost always have a bleeding disorder too. While there is some general information, the education sessions at women's weekend tend to focus on women's health needs.

This time we started with nurse practitioner Vanessa Minor and Haematologist Nicola Eaddy, both from the Auckland HTC, who talked about living with the haemophilia gene, von Willebrands, and other bleeding disorders. These sessions went very well, and prompted a number of questions and discussions. Vanessa, Nicola, and Lauren also sat on an experts panel at the end of the weekend. This allowed the women to reflect on what they'd learned and ask questions related to the sessions.

Outside of the specifically BD related health sessions, we also had a couple of more general wellness sessions. HNZ member Ashley Fowlie talked to us about mindfulness, and led us through a macrame workshop. This was a very relaxing session, which encouraged the women to really think about being in the moment, and making time in their busy lives for themselves, from time to time.

We also had women's health expert Linda Dear in to talk about menopause. This was a fascinating session from one of Aotearoa's leading menopause experts. With a wide range of ages present, it was lovely to see questions being asked and advice being given from within the group. This was by far the highest rated session of the weekend in the evaluations, and even generated a rallying cry for the weekend (if you know, you know).

However, this event was not all about education and wellness sessions. We also got out and about for some well-earned social time. On Saturday afternoon the women were able to head over the road to soak in the hot pools, go for a walk around the thermal terraces, or take advantage of the opportunity for some free time. As you can imagine, the majority opted for a nice long relax in the thermal pools. That evening we all enjoyed a burst of nostalgia when we dined at Cobb & Co. This was a great night out, which prompted a call for karaoke at the next edition of women's weekend!

The weekend finished with the experts panel on Sunday morning, before the women had the opportunity to reflect on the weekend as a whole, and offer their thoughts.

By all reports, this was a very successful event. Some knowledgeable sources even said the best ever! Of course, we couldn't have done this without the support of the HNZ community, the Board, and the people who put the time into organising it. Thank you to all involved.

We're looking forward to the 2026 edition already!

2024 Central region camp

This year Central held our Regional family camp in Napier on 4-6 October where we based ourselves at Kennedy Park. 64 attended, (33 Adults and 31 Children).

The programme included education sessions on bleeding disorders from Daryl Pollock, CNS at Palmerston North HTC and Hemi Waretini, Chair of HNZ's Board spoke about constitutional changes in HNZ and his own journey from attending camps through to his opportunities and roles within our organisation.

The children's creative crafts programme was most ably run by Ashley Fowlie and Amy Bollman while parents attended the education sessions.

Central's AGM preceded lunch on Saturday where we farewelled Tracy Nyhan as Board Chair, she will remain on our Committee as Regional Delegate. Ashley was appointed Chair and Amy Secretary. Two attendees also expressed an interest in helping the Committee.

Off-site activities included the interactive The Faraday Museum of Technology and The National Aquarium of New Zealand.

The facilities at Kennedy Park provided entertainment for all ages, the swimming pools were especially popular.

Saturday evening's games ensured lots of fun for all. Thank you Tracy and Greg Nyhan for running an excellent quiz and Bingo night.

Many thanks to our small Committee for their dedication and behind the scenes work in putting together such a great event. From all reports our best Regional Camp so far.

I would especially like to thank and acknowledge KiwiFirst in Central Region for sponsoring this event.

Lynne Campbell

Central Outreach

Around the regions

HNZ's Branch committees enable all our members to participate in the running of the organisation, and to connect with and support one another. Each Branch runs a number of events through the year, to help educate their local members, to make sure that support goes where it's needed, and to have a little bit of fun. Here's what they've been up to recently.

In the **Northern** region Darian has been busy with outreach. In the second half of the year he has run education sessions with schools and with Corrections. He's also been able to access tickets for RazzaMatazz, for A Midsummer Night's Dream, for a magic show, and for Armageddon, which were all enjoyed by local members.

The Northern branch also ran some events of their own. In August they headed to the Sweet Axe Throwing Co for some sharp-projectile-based fun. Then in December they went to Game Over Auckland to enjoy the go karts, a round of laser tag, and a spot of minigolf for their end of year event.

Meanwhile, the **Midland** crew have also had a couple of popular events for the second half of the year. In August they had fun rock climbing at Rocktopia in Mount Maunganui, accompanied by pizza and a good catch up. Then, in November, they had an end of year luging event at Skyline Rotorua. This event included a subway lunch and the opportunity to ride the gondola and enjoy the lakefront playground.

The Midland region were very sad to lose the services of long-time committee member and all-round awesome HNZ leader Tineke Maoate this year. Tineke and her whānau have moved to the far North. Midland's great loss is Northern's lucky gain.

In the **Central** region, outreach worker Lynne Campbell has also been keeping busy. She has attennded the Kapiti clinic, as well as the regular fortnightly Wellington clinics. She also went on tour, visiting members in Taranaki, Manawatu, and Wellington, including a lovely dinner with members in New Plymouth.

The Central committee are very good at keeping their members engaged, and this year has been no different. Since the last report the committee have run their biennial Family Camp in Napier, which was a great success with over 80 attendees! To round out the year they've held a Christmas celebration at the Coach House Museum in Feilding, complete with lunch and tractor rides.

The big news out of **Southern** was their regional camp in September. This was a fantastic event at Hanmer Springs, and marked the first regional camp in this region for over 10 years. Thank you to Hanmer Springs Attractions for supporting the Southern Camp!

Hamner Springs Attractions is North Canterbury's premium adventure activity base with qualified guides and a strong commitment to health & safety demonstrated by Qualmark Gold and AdventureMark accreditation. The activities available at Hanmer Springs Attractions include jet boating, bungy jumping, quad biking & buggy adventures, rafting, laser clay shooting and paintball set amidst stunning alpine scenery. Only 90 minutes' drive from Christchurch and situated outside the picturesque spa village of Hanmer Springs, Hanmer Springs Attractions is the perfect choice for action-packed fun with friends or family.

Hanmer Springs attractions
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PAINTBALL • LASER CLAY SHOOTING
QUAD BIKES & BUGGIES

https://www.hanmerspringsattractions.nz/

Vic has been working hard at outreach, including meeting up with the uni students before they finished for the year, and attending a conference aimed at defining equality vs equity. Southern also had an end of year committee meeting. There's now a fabulous and robust regional committee in the Southern region, who are all excited about fostering connections in the region.

Latest news

Clinically Translatable Approach to Gene Therapy in Hemophilia A With Inhibitors

Sheila Jacobs

Gene therapy offers the potential to cure hemophilia A via incorporation of the F8 gene into cells to attain the long-term production of therapeutic FVIII protein.

In murine models of hemophilia A with inhibitors, busulfan/ fludarabine or melphalan/ fludarabine preconditioning, followed by 2bF8 lentivirus (LV)-transduced hematopoietic stem cell transplantation (HSCT), can effectively and safely initiate sustained platelet factor VIII (FVIII) expression and generate immune tolerance, according to findings from a study published recently in the Journal of Thrombosis and Haemostasis.

The X-chromosome-linked recessive bleeding disorder hemophilia A is associated with a deficiency in FVIII. Although protein replacement therapy is effective in patients with hemophilia A, those with more severe forms of the disease go on to develop anti-FVIII inhibitory antibodies (ie, inhibitors) following FVIII infusion. This, in turn, can have a considerable impact on the outcomes of clinical care, with inhibitor development against FVIII remaining a key concern.

In the current analysis, the researchers sought to assess the efficacy of busulfan/fludarabine and melphalan/fludarabine preconditioning in platelet gene therapy for hemophilia A with inhibitors. It is recognized that gene therapy offers the potential to cure hemophilia A via incorporation of the F8 gene into cells to attain the long-term production of therapeutic FVIII protein.

The safety and durability of adeno-associated virus-mediated liver-specific FVIII gene therapy have met with some concerns. Recently, more advanced transplant conditioning regimens have been developed to improve patient outcomes. In fact, in reduced-intensity conditioning, an alkylating agent—that is, busulfan or melphalan—plus the nucleoside analog fludarabine is the predominant therapeutic regimen used for HSCT in a number of disease models.

The investigators used busulfan/fludarabine and melphalan/ fludarabine to condition mice with hemophilia A that had been preimmunized with recombinant human FVIII. At the same time, an optimal 660cGy total body irradiation (TBI) was used as a control preconditioning regimen. Transplantation of 2bF8LV-transduced HSCs was used to establish platelet FVIII expression. FVIII assays, tail bleeding tests, and fluorescence-activated cell sorting were used to analyze the mice.

Results of the study demonstrated that busulfan/fludarabine, but not melphalan/fludarabine, allowed for successful 2bF8LV gene therapy. In fact, 20 weeks post-HSCT, all of the recipient mice maintained more than 55% of donor-derived leukocytes—that is, in both the busulfan/fludarabine group and the 660cGy TBI control group. Levels of leukocyte chimerism were 83%±9% in the busulfan/fludarabine group compared with 72%±4% in the TBI control group, with no significant differences observed between the 2 groups.

Comparable copy numbers of 2bF8 cassette and platelet FVIII levels were seen in both groups. “The bleeding phenotype was rescued in 2bF8LV-transduced recipients.” All of the untransduced transplanted control mice, on the other hand, produced inhibitors. Thus, immune tolerance was established in 2bF8LV-transduced primed mice with hemophilia A under busulfan/fludarabine conditioning.

“Busulfan/fludarabine preconditioning allows for successfully introducing platelet-FVIII expression to restore hemostasis and induce immune tolerance in primed hemophilia A mice, suggesting that this approach is a promising clinically translatable strategy for gene therapy of hemophilia A with inhibitors,” the authors concluded.

Source: https://www.hematologyadvisor.com/news/hemophilia-clinically-translatable-approach-gene-therapy/

Emicizumab as a Potential Treatment Pathway for Select Hemophilia B Cases

Lisa Kuhns, PhD, MD

New research highlights the potential of emicizumab, traditionally used to treat hemophilia A (HA), as a promising alternative therapy for certain hemophilia B (HB) genotypes, according to a study published in Blood.

HA and HB are X-linked bleeding disorders caused by reduced activity in clotting factors VIII (FVIII) and IX (FIX), respectively, and are traditionally managed by intravenous factor replacement. Emicizumab, a bispecific antibody that facilitates clotting without requiring FVIII, has transformed HA management by providing an effective, subcutaneously administered treatment. In contrast, HB continues to rely on intravenous FIX infusions, as subcutaneous non-factor therapies (NFTs) have faced challenges due to thrombotic complications.

“Although described as an FVIII mimetic, the emicizumab-FIXa complex is biochemically distinct from FVIIIa-FIXa,” explained Kyumin Lee, Division of Hematology, Department of Pediatrics, Children’s Hospital of Philadelphia, Philadelphia, PA, and coauthors. “We hypothesized that these differences can be leveraged to repurpose emicizumab as a potential treatment for certain HB F9 genotypes.”

Researchers screened 105 HB-causing FIX variants and identified 41 that showed improved clotting activity when emicizumab was present. Specifically, they found that HB variants with loss-of-function (LOF) mutations in the FIX gene could benefit from the procoagulant effects of emicizumab, as demonstrated in laboratory tests and select patient samples. For instance, clotting times and thrombin generation significantly improved in patient blood samples with the FIX-I397T variant when emicizumab was added, indicating that emicizumab may support clotting activity in specific HB cases.

“Combined, these data demonstrate the potential of emicizumab and likely other FVIII mimetics to improve the procoagulant activity of selected HB-causing FIX variants,” the study authors concluded.

Although emicizumab doesn’t completely replicate FIX function, it could serve as a viable NFT for individuals with select F9 genotypes, offering an alternative to intravenous therapies. This research represents a promising step toward expanding treatment options for HB and underscores the need for further clinical trials to confirm the safety and efficacy of FVIII mimetics in this novel application.

Reference: Lee K, Chau JQ, Suber YB, et al. Enhanced procoagulant activity of select hemophilia B causing factor IX variants with emicizumab. Blood. 2024;144(11):1230-1235. doi:10.1182/blood.2023021944

Source: https://www.hmpgloballearningnetwork.com/site/frmc/news/emicizumab-potential-treatment-pathway-select-hemophilia-b-cases

Assessing the factors affecting the accessibility of primary dental care for people with haemophilia

Kitti Sipos, Ildikó Márton, Marianna Móré, Attila Csaba Nagy, Csongor Kiss

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Attila Csaba Nagy and Csongor Kiss contributed equally to the work as senior authors.

Abstract

Introduction

Patients with haemophilia (PWH) often have difficulty accessing dental services.

Aim

To determine the accessibility of dental care for PWH and to examine their perceptions of how coronavirus type-2 (CoV-2) disease (COVID-19) has affected their ability to access dental treatments following the pandemic.

Methods

The questionnaire survey was conducted between July 2022 and December 2022 at haemophilia treatment centres in Hungary. Variables with statistical significance (Pearson’s Chi-squared test; $p < .05$) were included in logistic regression analyses. Least absolute shrinkage and selection operator (LASSO) regression was used as a machine learning technique to identify the most predictive variables.

Results

Twenty-one percent of the sixty-eight participants reported that they had been refused dental treatment, mainly in primary care (86%). Dental refusal was influenced by infectious disease (OR: 4.48, CI: 1.14–17.69) and previous dental bleeding complications (OR: 4.23, CI: 1.10–16.27). There was correlation between dental visits and having a permanent dentist or receiving oral hygiene advice (OR: 9.95, CI: 2.86–34.62 and OR: 3.84, CI: 1.09–13.58). Participation in an oral hygiene consultation increased patients’ satisfaction with their dental care (OR: 6.28, 95% CI: .71–55.88). Twenty-eight percent of patients had experienced difficulties since the start of the COVID-19, but 84% had visited their dentist at least once between 2021 and 2022 ($p = .002$). Nevertheless, 16% of respondents went for only the most necessary treatments due to pandemic.

Conclusion

Refusal of dental care was high among participants, especially in primary care. The COVID-19 pandemic has exaggerated the difficulties of PWH in accessing dental treatment.

Highlights

Patients with haemophilia (PWH) have difficulty accessing dental care, and the coronavirus type 2 (CoV-2) disease pandemic (COVID-19) has created a new barrier.

The study revealed a high prevalence of dental care refusal (21%), particularly in primary care (86%).

This 2022 survey found that 28% of patients experienced difficulties since the pandemic started and 16% only sought necessary treatments.

Source and full text: https://onlinelibrary.wiley.com/doi/10.1111/hae.15124?af=R

Giving Choice a Voice: Commentary on Development of the World Federation of Hemophilia Shared Decision-Making Tool

Leonard A. Valentino, Kate Khair

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ABSTRACT

The shared decision-making (SDM) tool developed by the World Federation of Hemophilia (WFH) provides a clear and concise overview of the process by which people with haemophilia (PwH) can collaborate with their healthcare professionals (HCPs) to engage and arrive at a therapeutic decision. This tool will be useful for all people with all bleeding disorders, not just PwH.

1 Commentary

The shared decision-making (SDM) tool developed by the World Federation of Hemophilia (WFH) and described by Coffin et al. [1] provides a clear and concise overview of the process by which a person with haemophilia (PwH) can collaborate with their healthcare professionals (HCPs) to engage and arrive at a therapeutic decision [2].

The tool provides a framework to facilitate a discussion between PwH, their caregivers and HCPs on the available prophylactic treatments by providing education on the risks, benefits and alternatives while helping to elicit answers to key questions regarding current disease impact and current and future treatment preferences with a goal of optimizing outcomes and satisfaction. Educational fact sheets and videos supplement direct interactions between the participants. An online platform and a printable workbook are available to support the conversations.

SDM functions optimally when there is evidence-based information which can be shared with the PwH along with information based on the HCPs knowledge and experience but without bias or predetermination. The PwH has an integral role in the SDM process in which she must articulate the values and preferences that are important and what challenges are currently impeding an optimal outcome. A discussion and collaborative process ensures in which both the PwH and the HCP have the opportunity to express their thoughts and feelings regarding the various options available. It is important not to discount one or more options because of lack of familiarity, knowledge or bias. Both parties must be willing and empowered to seek new information and gain new knowledge. In addition, complacency with the current treatment regimen must be avoided and both parties must maintain an open mind as to new options. It must, however, be remembered that new is not always better. Cost and financial burdens must be taken into consideration along with the likelihood of adherence to a new therapy and balanced against historical patterns of adherence. SDM functions optimally where there is a full palette of options available to the PwH and the HCP to debate and deliberate the expected outcomes and risks with the preferences and values of the PwH.

SDM is predicated on the fact that there is a choice between treatment options. In many low- and middle-income countries there is either no treatment or limited options [3] available making the decision process challenging. PwH in these countries are desperate for any care and treatment and often rely on humanitarian assistance from the WFH [3].

Unfortunately, SDM requires a degree of knowledge about their disease and the treatment options available, and health literacy and numeracy among PwH is low [4]. HCPs must be willing and capable of providing basic education to PwH and their caregivers to enable a fruitful discussion which should be tailored to ensure that those with low levels of health literacy or socio-economic disadvantage are not excluded [5]. Furthermore, HCPs must be willing participants in the process and serve as facilitators relinquishing their role as primary decision-makers and deferring to people who live with haemophilia. Many HCPs are not familiar with SDM and will require their own education on the process and that of motivational interviewing. They must also become aware of their own biases and overcome them along with the lack of familiarity with newer treatment options to be able to provide a truly comprehensive perspective on the options available along with their risks, benefits and alternatives.

Haemophilia has an estimated global prevalence of about 830,895 [6] dispersed across all continents. As such, multilingual and cultural considerations are necessary along with education of PwH and HCPs for successful implementation of the tool.

SDM is rooted in people-centric care which has been promoted by the World Health Organization and numerous specialty societies [7, 8]. This type of care is guided and informed by patients' goals, preferences and values and is built on trust and a commitment to long-term health and well-being. It focuses on quality of care and achieving optimal outcomes, emphasizes HCP performance and enhancing the patient experience but must also include consideration of risks and consequences of different treatment options. Practicing SDM in the context of haemophilia care takes advantage of the integrated comprehensive care available from haemophilia treatment centres (HTCs) [9] across health systems, providers and care settings and is ideal for managing chronic and complex conditions like haemophilia.

For the WFH SDM tool to be successfully implemented, all treatment options must be presented in a fair and unbiased fashion so that PwH and HCPs can have an open dialogue. It is heartening to see that the WFH SDM tool includes all therapeutic options and not only gene therapy for which it was initially developed to support.

We admire the WFH SDM tool for its application to SDM for the treatment of PwH—we must, however, consider SDM for all people with bleeding disorders—not just haemophilia—as new therapeutic options become available and offer treatment options for them.

Source: https://onlinelibrary.wiley.com/doi/10.1111/hae.15133?af=R

Upcoming events

6 - 9 February 2025

National family camp

Ngāruawāhia.

10 May 2025

HNZ office opening

Wellington.

31 October - 2 November 2025

Adult wellness weekend

Wellington.

January 2026

Teen & youth camp

Auckland.

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

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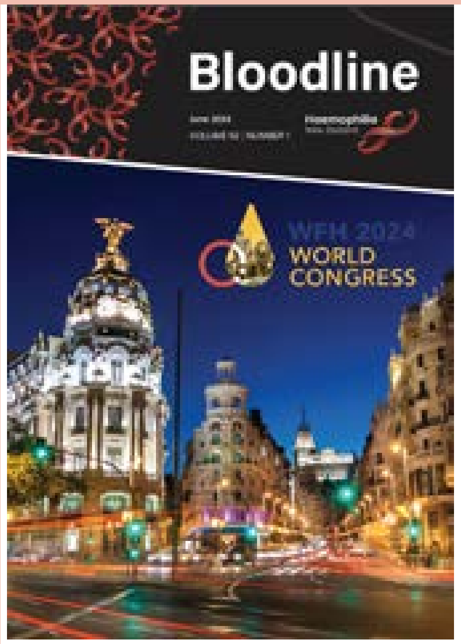

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