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Magazine of the Haemophilia Foundation of New Zealand. Volume 46 Number 2

Bloodline

Haemophilia Foundation of New Zealand Inc.
HFNZ

Regions and Groups Reports «
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The H Word

We are virtually there. Really, we are. From mid-June, the office has become virtual. Our staff are floating somewhere in the cloud. What does this mean for you? If I hadn't mentioned it, you may not even have noticed. The office-based staff now join the outreach staff in being a virtual team for now. As a member, you will continue to receive an uninterrupted service!

What does this mean for the property purchase? You may recall that last year we began a property search so that we can relocate the HFNZ national office to Wellington, with endorsement later that year at our annual general meeting. This has not changed. The property fund remains intact, and we are working through realising a generous bequest, which will assist us greatly in finding the right commercial property, as well as providing more for our members. We can only do this once, so we have to get it right. This decision is about both appropriate investment, and finding the right home for HFNZ.

While this is an important focus right now, the National Council have noted staff turnover levels, and our CEO is working to recruit a Southern and Midland outreach worker as soon as possible.

I would like to thank Joy for her many years of service to our Midland community, and wish her well with her future role. Joy was with HFNZ for eight years. Thank you Joy!

In May, a contingent of 15 members went to the World Federation of Hemophilia World Congress in Glasgow. I hasten to add that we made funding available for five, with the remainder self-funding flights and accommodation. I think it is heartening that so many members were willing to give up personal time and their own expense to learn more. You can read more about what's new in the bleeding disorder world in this issue.

Remember that while many of you have completed the member survey, you can always write to me directly if you have any feedback.

Here's to a short winter and a long summer.

Deon York
HFNZ President

2018 WFH Congress: Glasgow

The 2018 WFH World Congress was an opportunity to bring together members of the world patient community, along with a wide range of professionals including clinicians, social workers, outreach workers, supporters, and advocates. HFNZ was lucky enough to have 15 people represent it this year. President, Deon York attended in his WFH capacity, while six other members and staff were fully funded by HFNZ. The remainder were self-funded, with HFNZ paying their registration. Our people presented three posters, spoke in Congress sessions, and have reported here on the new and interesting things they learned.

A bleed or not a bleed; that is the question

BY TUATAHI PENE

Pamela Narayan chaired the presentation, with five speakers from multi-disciplinary backgrounds, covering the differences between the following questions:

- How we can diagnose what is an acute joint or muscle bleed?
- How is this different from an acute or chronic bleed?

They all agreed that not every bleed is the same, and that understanding the differences will lead to better treatment.

The first speaker, Dr Radoslaw Kaczmarek, spoke about his own experiences living with severe haemophilia, and how he had managed his own self-assessment of bleeds to best identify what course of action was needed to remedy them.

A background to how treatment had been undertaken in Poland highlighted the many barriers Dr Kaczmarek had faced in his early years of factor transfusions. Although prophylaxis became available in the 90s, the mind-set around how bleeds were to be treated still led to injuries in patients, such as knees, ankles, and elbows, which of course resulted in loss of mobility and strength over time.

This did not deter Dr Kaczmarek's motivation to self-improvement. In his early teens he started his own assessment on how best to treat himself, with the attitude of strengthening his body. He had undertaken the so-called 'nerdy approach', where he experimented with light exercises, progressing to resistance training, and finally to compound movements.

These daily workouts resulted in developing a better understanding of his own body; and he found an answer to the question of "What difference does it all make in the way I tell bleeds from arthropathy flares?" Dr Kaczmarek noted that the sensation would differ between a bleed caused by activity, and the arthropathy flares that occur from past bleeds where it could damage the joints. By experiencing the sensation of the differences, he was able to generate workout programmes best suited for his needs. Working out 4 to 5 times a week, his focus now is compound movements to strengthen muscles.

Bojan Pirmat stood next as the second speaker. Similar to Dr Kacmarek, he spoke about his journey with mild haemophilia, and the way his experiences have led to a deeper understanding of his own bleeds.

Bojan spoke of his days living in a big city in Canada, where the line on treatment involved how not to focus on a single bleed, but on how to help the patient as a whole. A team of multi-disciplinary experts aid the patient by discussing the cause of the bleed, and, together with the patient, analyse and draw conclusions to help find the best approach of treatment. Bojan, as a mild bleeder, did not have many significant bleeds to cause worry, and, through being a less active child, he had accumulated many minor bleeds. The team of experts that was working with Bojan noticed the onset of possible arthropic bleeding, and so recommended resistance-training exercises. Over a short period, the exercises caused frequent pain. Not knowing if the pain was the result of exercises or bleeds had caused Bojan a lot of grief. After a while, the exercises became less frequent, and, in the end, stopped all together.

A review of the regimen undertaken suggested that it might have been best to find a balance of workout exercises that caused less pain and helped maintain achievable goals.

The third speaker, Dr Nathalie Roussels, spoke about behaviour in response to pain. Chronic pain in bleeds can lead to confusion in diagnosing the best form of treatment for the patient. Finding solutions to chronic pain comes from understanding behaviour, the link between beliefs about pain, and the idea that 'it's all in your head'. The healthcare provider and the patient discussing the sensation of the bleed can help find the best solution for pain treatment.

It is important to understand the difference between a patient's 'normal' behaviour and their 'pain' behaviour. The link between beliefs and behaviour can affect how an individual perceives pain, by understanding the sensation caused by pain, the assessment by the patient and their health care provider can formulate the best procedure for treatment.

Karen Strike was the fourth speaker of the seminar. She spoke on her perspective of current and future management of acute and chronic bleeds. Over her professional career as a physiotherapist, she had comprised a cohort of information from patients suffering from a range of ailments; bleeders and non-bleeders.

Why is it so hard to tell if there is a bleed? What can health care professionals do to get an accurate assessment on the injury? A relatively new technique called Point of Care Ultrasonography has seen great

results in bleed assessments. An internal view of the injury is projected on screen to show where the problem areas are, and, from here, a proper diagnosis can be made.

There are limitations to consider, one is that it is operator dependent: risk of misdiagnosis is high when used improperly or by inexperienced practitioners. That means appropriate training, competency assessment, and quality assurance is important. There is a need for valid and reliable protocols for acute joint and muscle bleeding.

The fifth and last speaker, Dr Brian Feldman, spoke on the Assessment of Bleeding – What is the role of patient reported outcomes?

A patient can only report to the health care professionals about what he/she already knows. A prior knowledge on what a bleed feels like can be helpful, but at times misleading. When a patient is confused about what they are feeling, or finds it hard to describe the sensation of bleeds, they rely on the healthcare professional to provide the best words best to describe what it is.

A patient-reported outcome (PRO) is a health outcome directly reported by the patient who experienced it. It stands in contrast to an outcome reported by someone else, such as a physician-reported outcome, a nurse-reported outcome, and so on.

PROs should not be confused with patient-centred outcomes. The latter implies the use of a questionnaire covering issues and concerns that are specific to a patient. Instead, patient-reported outcomes may or may not be of concern to the patient.

A Personal Experience of Congress

BY NICKY HOLLINGS

It's not very often that you get the opportunity to be brought to tears by a psychological/sociological presentation on the history of women and bleeding. Our very own (yes let's claim her as our own) Claire McClintock gave a brilliant presentation on the impact of being a woman with a bleeding disorder. How the different beliefs and values have led to women having been restricted from temples, shrines, and religious ceremonies, where they have been isolated and secluded. However, in some cultures, it is when women are thought to be at the height of their powers; menstruation is a time to rest, to be with oneself, and a time when women are sacred. Please watch the presentation when it is online, I can't give it justice.

It was fabulous to see our youth on the world stage talking about their twinning adventure. The beauty for me was that our twinning team is our youth; they are doing the work, making it happen, walking alongside the Nepalese youth to produce the outcomes that Nepal wants, rather than coming in as a country that knows best, or having older team members discussing what youth need. Our Youth Looked Great!

Living in a developed country where our members get treatment by right, it was a humbling experience to hear Megan Adediran from Nigeria and Asraf Caunhuye talk about their fight to be heard, so that their sons can have treatment, and how they have developed their national organisations.

Megan received the WFH President's Award, from all her efforts for the Nigerian Haemophilia Community. I was so happy and excited for her, as I had been lucky enough to hear of her work and to meet her previously at NAACHO.

Then there were the members' stories; Jack Grehan being brave enough to have gene therapy, Andrew Selvaggi, who found himself again through exercise and self-care. The list goes on.

A number of men stood up asking for a voice at congress. These men are living with HIV, and they were concerned that they are becoming forgotten members of the Haemophilia world. This made me wonder, is this happening in New Zealand, and if it is what do our members want and need for this to change?

Seeing most of our team every morning (those that stayed in the same hotel) for breakfast was a joy. Meeting in the morning to see how their evening had been and what they planned for the day was a lovely way of connecting. Then, catching up with others in between sessions, or getting to see them present, it was a privilege to be part of HFNZ.

People have often asked me what my job is like. Yes, at times it is working with people that are in crisis and that can be challenging. However, what I say is that I get to work with kind, caring people, who are wonderful parents and men and women, and, at Congress, I got to do this for a week in Scotland.

Caring for carers

BY TE WHAINOA TE WIATA

Often, within the bleeding disorder community, focus is solely on the patient and their needs as identified by the health professional. Yet, the majority of the time these needs are carried out by the main caregiver or guardian. And, although their sacrifice is acknowledged, these acknowledgements are often implicit, and as a result the supports for the caregivers go by the wayside. This session was an informative presentation, shedding light on the fact that science and education towards the patient is improving every year, yet basic support for caregivers has not advanced at all. Ongoing stigma, anxiety, and stress, triggers the reality that these people want their child to live a normal life, but they (the caregiver) fail to do so themselves. This is the need that the speakers addressed while providing solutions as to how this can be achieved.

Vice president of the Serbian Haemophilia society Tatjana Markovic spoke on the role of the carer at home, for example:

- Parent
- Siblings
- Extended family
- Friends.

These are the people who commit their time and sacrifice a portion of their life to looking after their child or loved one. Speaking from the view of a mother and carrier of the Haemophilia gene, stigmatization was the highlight of Markovic's talk. Trying to deal with the outside perspective that people have about someone with a disability or disorder of some sort. As when a stigma exists, the entity or person it is aimed towards is considered to be outside of the "normal" way of life. Thus, follows the constant drive for inclusiveness, making sure their loved ones do not feel the stigma people hold, while on the other hand, ensuring that those that have formed a perspective are informed enough to dismiss the idea of a stigma, it can often feel like a road with no end.

Markovic then states that the level of stigmatization depends on four factors:

- The level of treatment in your country
- Support of medical and social institutions
- Support of family and friends
- Support of the immediate environment and society on the whole.

A lack in any one of these areas produces a harder time for the carer and associated members of the immediate and wider family. This produces a question around identifying common issues and hardships for caregivers and the surrounding family.

Peg Geary, project manager and social worker at the Boston Hemophilia Center, presented a recent survey that took place in Boston. The idea was to identify common areas of hardship for caregivers, determine options and plans, and then decide on the best response. Questions included:

(**Note:** These questions are completed by outreach workers based on their observation)

- Physical tasks related to care (infusions, medical appointments daily tasks etc...)
- Emotional issues (current physical condition, stress and anxiety about condition etc...)
- Finance (financial and insurance concerns due to caring for loved one instead of working)
- Social isolation (due to time and other commitments)
- Personal exhaustion (Through time caring and lack of support)
- What support would help lift the load? (Family assistance, professional assistance, insurance, education etc...)

The above presents the type of observation the outreach workers are looking for and results generally indicated that these carers scored in the higher percentile for needing support in these areas. Other questions included:

- What is lacking in the caregiver tool box?
- Rate the support of outreach worker.
- How does a caregiver take time out?
- Are there differences in practice between different ethnic groups?

All of these questions presented a clear picture to Geary and her team for future support towards caregivers and better preparation for the outreach workers.

Clinical Psychologist Dr Gráinne O'Brien, then presented what has been taking place in Scotland recently with regard to haemophilia care. Firstly, classifying anyone who helps with care as informal caregivers; relatives, partners, friends and neighbours, and those who are paid to give support and care as formal carers. Dr O'Brien breaks the world of the patient down into four areas:

- Family
- Classroom
- Religious setting
- Peer group.

These four areas are what is seen as the world of the patient, however in their line of work due to resources they are generally only able to tend to the family setting. Yet, this opened up the whole thought of the formal caregivers even being the classroom teachers and how under prepared they are in unknowingly taking on the task of a child with a bleeding disorder in class. It is acknowledged that there is need for such issues to be addressed, yet, finding the most efficient path is the hard task that Dr O'Brien and her team are looking to now. Thus, a survey much like the one presented by Peg Geary, to help identify what the needs are, where support is lacking and what options exist.

Finally, Dr O'Brien, spoke about a caregiver tool box, which would ideally include:

- A peer group
- A helpline
- Advisors
- Education (coping mechanisms)
- A place for time away.

In conclusion, the session presented and broke down what is lacking when it comes to the informal carer and the stresses that come with stepping into the caregiver role. It also provided an avenue to start talking about support for caregivers. Overall, the main theme of the session was to look after those who are looking after you as a patient. It provided a very holistic and practical approach to attending to the needs of carers and the hope that we can all take that time to acknowledge them properly.

In closing, the conference itself was for lack of a better word 'awesome'! Seeing and hearing about the innovations and listening to those at the forefront of the research is always very inspiring and recharges the desire to help. Being there with quite a large contingent from Aotearoa made it much better, where we were able to give and receive feedback, and hear about other sessions that some were unable to attend. All in all, it was an amazing experience, it was very exciting to hear about all the new innovations and it was great to touch base with old friends from our global community. Thank you HFNZ for allowing the experience to take place.

Ngā mihi aroha ki a koutou katoa.

Child to teen, what does it mean?

BY LAURA WATSON

There were several keynote speakers for this session. The session explored how healthcare providers, patients, families, and caregivers can work together to offer teens a smooth pathway from child to adolescent and adult services. It also suggested how treatments and care could be optimized while maintaining good communication, trust, rapport, and allowing special relationships to continue to develop and grow for the benefit of the patient and their support network.

Brittany Savage is a bleeding disorder transition nurse from the Indiana haemophilia and thrombosis centre. Nurse Savage describes a nurse's model for transition from paediatric to adult services.

For young people, transitioning into adult services is a tentative time, as they move from a service that is more family centred to a service that is more patient centred and self-managed. It is an important time for a young person as they learn to self-manage. However, there can be barriers and consequences if medical professionals do not handle the transition correctly. If successful, this can be a smooth and empowering time for young people. Potential barriers include:

- Sense of loss or abandonment
- Parental anxiety/fear of unknown
- Paediatric provider reluctance to transfer
- Finding an adult provider
- Young adult lacking self-management skills.

In Nurse Savage's programme, the transition begins early, between 14-16 years. The idea of a multidisciplinary approach is central to the programme, as is individualised care, as one size does not always fit all. Parents and caregivers also need to go through the transition of changing from a primary caregiver to a support person. Engagement and motivation are key factors as it is important to consider your audience. When planning a transition for a young person there are some important things to consider:

- Discuss the patient's personal goals and priorities related to their healthcare
- Prepare a medical summary and emergency care plan for upcoming transfer of care
- Plan for timing of transfer and identification of an adult provider
- Prepare the family
- Changes related to decision making and consent to release health information
- Expectations related to their adult provider team
- Provide orientation information
- Communicate with paediatric provider prior to first visit

- Determine young adult's preferred mode of communication
- Assess the young person's ability to provide self-care
- Review any transfer of care documents and obtain required consents.

The biggest takeaway from Nurse Savage was that transition is not merely a transfer of care. Start early to achieve the best result possible, a multidisciplinary approach is optimal, transition looks different for everyone - individualise, parents and caregivers should be involved in the process and utilise available resources.

Zikra A. Aikhayal is a dentist at King Faisal specialist hospital and research centre.

Aikhayal describes paediatric to adult dental care: an example of transitional service change.

Aikhayal echoed many of the key ideas from Nurse Savage's presentation. The time of adolescence is the perfect time to set up for adulthood, and the process is a dynamic one that seeks to meet the needs of the individual. The transition for oral health begins between the ages of 14-16 years. The transition of oral health for young people and their parents and caregivers is similarly not without barriers. The psychosocial burden that begins with the parents is gradually transferred to the young adult. Self-management becomes very important and can be a lot to handle for a young person, and dental care is the most common unmet need. It is also a unique time for a teenager as oral hygiene gets worse due to diet, and dental visits begin to drop. Other barriers such as a shortage of dentists for haemophiliacs and lack of reimbursement only compound the problem. Aikhayal believes that, for haemophiliacs, prevention is key and, again, a multidisciplinary approach is optimal. Finding a dental home is important; somewhere a patient knows to go. Empowering teenagers with responsibility and a place to go is key.

Natalia Andrea Arroyave Botero social worker, professor - Universidad de Antioquia, consultant for the development and the strengthening of comprehensive care models of haemophilia in Colombia. "Understanding the stages of transition".

When we consider the reality of someone with haemophilia, we need to consider that it does not just concern the patient, it does not just affect the body, and it is more than pharmaceutical treatments. Botero, along with the speakers before her, also champions a multidisciplinary approach, which works best by having a comprehensive care team. Botero suggests using a holistic view by looking at the whole picture to understand all the things that form the world of a person, not just the diagnosis of that person. A risk analysis tool helps the process of a multidisciplinary approach by generating a detailed profile of each patient according to their age and course of life. This can help to determine an intervention plan articulated by the specialists of the program, the clinical, care, educational, and administrative actions that respond to aspects of risk that require intervention, and the identified protective or success factors that can be enhanced. The overarching statement of Botero's presentation suggested that education and an individualised approach can lead to an autonomous and empowered patient. Similarly, in a community environment, people with similar experiences can come together to expand each other's knowledge.

Continuity in Quality of Life

BY LAURA WATSON

This session had several key speakers.

All too often only positive stories and outcomes are presented to peers and patients at conferences. In reality, clinical practice is a rich and varied storyboard of individual approaches, personalities, patients, and lifestyles. This session presented some difficulties identified by experienced treaters and patients, explained how they felt about them at the time, and reflected on the decision-making processes chosen to address the issues. This session focused on the hard road and hard work that sometimes goes into reaching positive outcomes.

Debra Pollard is a Lead Nurse at the Katharine Dormandy Haemophilia Centre, Royal Free Hospital, in London. Pollard shared the story of Jamilla, who has Bernard Soulier Syndrome. The case study followed Jamilla from early childhood into adulthood. Quality of life for Jamilla was good, and the management of her condition was relatively simple in her preteen life. However, with the onset of menstruation Jamilla's quality of life began to decline. Jamilla began to have constant bleeding, which meant frequent hospital visits and consultations. A bone marrow transplant was offered as an option. However, further discussion with Jamilla and her family about a bone marrow transplant and the associated risks; like fertility and body image concerns, and a fear that the risks of a bone marrow transplant outweigh living with Bernard Soulier syndrome, led to a decision not to have the transplant. The concept of shared decision-making is central to Jamilla's story. The benefits of

shared decision making include:

- Improved knowledge and understanding
- More accurate risk perceptions for patients and families
- Greater confidence with decisions
- More participation
- Better treatment adherence
- Improved confidence and coping skills,
- Improved health behaviours and more appropriate service use.

At 20 years old Jamilla felt like she could no longer go on, as her periods were destroying her quality of life. Along with her team and her family, previous discussions were revisited to ensure fully informed decision-making. Importantly Jamilla's family were invited back to be included in the process, as cultural and family factors affect decision-making. Jamilla accepted the risks as she felt her life wasn't worth living anyway, and, after her family accepted her decision, she was referred to the bone marrow transplant team. Once she received a donor bone marrow transplant, her periods reduced to three days and her quality of life greatly improved.

Pamela Narayan Consultant Physiotherapist at Lakshmi Hospital Hyderabad, in India. Narayan shared a case study of an 11-year-old boy with severe haemophilia A. His mother initially made contact through a WhatsApp group that Narayan had created. The boy had severe pain on his right side and had been in bed for 45 days. He was seen by a haematologist, neurologist, and an orthopaedician, and had investigations of his blood, and an X-ray and a MRI of his pelvis and thigh. Everything came up clear so he was diagnosed with a bleed in his upper thigh, and given factor on demand.

His parents thought he was avoiding school and being lazy. When Narayan saw him, he had become very uncooperative and difficult to deal with at home; he still had pain in his right thigh preventing him from sitting, standing, or walking. Even lying down was difficult for him. He had previously been a good student and liked school, but had become very angry with people as no one believed him. By the time Narayan saw him it had been 90 days and he had a severe fear of doctors. Narayan diagnosed a bleed in the Tensor Fascia Lata of the right thigh now partly fibrotic and so very painful. Her treatment was:

- Counselling and building bridges
- Convincing the boy that they believed him
- Convincing the parents that there is a physical problem and
- Physical therapy, final clinical diagnosis, & a treatment plan.

Over four weeks a complete transformation took place. The boy became a full participant in the treatment affecting his care, and began setting functional goals. The lessons learnt from this case study:

- Believe in the child
- Treatment must be led by clinical findings supported by diagnostic tests not vice versa
- Treat the whole family
- Focus on the development and use of soft skills to better practice
- Team work
- Empower the patient even a child
- Be forthright… a health care professional is allowed to say 'I don't know" 'know' the patient (and their family)
- Avoid distancing yourself as a treater from your patient
- Collaborate.

Andrew Micheal Selvaggi, from Melbourne Australia, shared his inspiring story of living with haemophilia. He was diagnosed at 15 months old, and with a an inhibitor at 2 years of age. At 7 years old he was placed in a wheel chair and left untreated for 11 years. He was bleeding 3-4 times per week and was recommended a knee fusion. His schooling attendance was about 30%, and he had a BMI of 34 at 95kg.

Selvaggi knew that things would not get better unless he made the decision to change, so at 17 he started riding 1 minute a day on an exercycle, slowly increasing until he ended up doing this 3 x 45min per day. Selvaggi learned that a stronger body and muscles supports healthier joints, and leads to less bleeding. He lost 30kg, began weight training four times per week, cardio 3 times per week, and became a qualified personal trainer. He was working full time and now only bleeding 1-2 per month.

Moving more can mean increased risk, but less movement also has increased risk. Emotional pain can manifest itself as physical pain, so it is important to push through, plan for the future and how you can be your best self. With youth comes learning. What Selvaggi would have done differently was:

- Accepting physical reality
- Changing focus and incorporating his passion into his daily life to remain motivated.

Lastly, he left the audience with a powerful message: If you have access to treatment – do it! So many countries don't – It's your duty.

From the Roots to the Treetops:
HFNZ Advanced Youth Leadership Training

Karl Archibald, Programme Facilitator.

Over Waitangi Weekend, 03 - 06 February 2017, HFNZ held its first ever Advanced Youth Leadership Training (ALT) weekend in the central North Island of New Zealand.

What was it all about?

ALT followed on from the HFNZ Youth Leadership Training weekend run in August 2014, and was targeted at specially-selected young people from around New Zealand. Also included was a member of the Cambodian Hemophilia Association (CHA), which has twinned with HFNZ. These 18 - 30 year olds have stepped up into leadership roles in their organisations, and have grown their involvement in their bleeding disorder communities. The ALT programme was designed specifically to enhance their leadership skills.

"The advanced leadership weekend was a unique and beneficial time. I developed conflict resolution skills, and a deeper understanding of how HFNZ interacts with local and overseas organisations."

What was involved?

The challenges started the moment they landed in Auckland on day one. Split into two teams, the groups had to problem-solve their way to the first night's base, 192 km south. Here the leaders learned about HFNZ's roots, history, and sacrifices. Together they shared personal stories, learned what it is like to have a bleeding disorder in a developing country, about not-for-profit leadership for tomorrow, social enterprise, and about building on the foundations laid down by others before them.

On day two the teams had to make their own way 150km east to Rotorua, where Executive members of the HFNZ National Council discussed risk management, what the Foundation is doing today, and what could happen tomorrow.

Day three saw the participants learning some higher-level advocacy strategies, and discovering some ways to manage group conflict.

The final day covered the global youth scene, some planning for tomorrow, and setting SMART goals.

"Invaluable opportunity, jam-packed with experiences/activities to develop the young people of HFNZ to step up and lead the foundation forward now and in the future."

What were the Outcomes?

There have been some great outcomes from the ALT Weekend. There is a higher level of engagement, with 13 foundation related goals or projects planned or completed. These include running local events and workshops,becoming a committee member, further leadership development, and greater advocacy through increased board involvement.

Overall, ALT has provided greater knowledge, increased confidence, and better understanding of the impact that each person has on the future of the bleeding disorder community.

Haemophilia Foundation of New Zealand Inc. HFNZ

The HFNZ Advanced Leadership Training Weekend was supported by unrestricted grants from CSL Behring Ltd. and the Global Blood Disorder Foundation.

Current and future laboratory issues

MĀHIA NIGHTINGALE-PENE

This session was about laboratory sciences, and had three different presenters with three different topics.

- Paediatric haemostasis: what is normal? – Pierre Toulon
- Effects of snake bites on haemostasis – Gary Moore
- Diagnosis of VWD – which activity assay to use? – Franscesca Stufano

This report will cover the presentation by Gary Moore about the, 'Effects of snake bites on haemostasis'. I won't be reporting on the other two because I simply didn't understand them.

Gary Moore provided an overview of the history of snakes, and what they are. Snakes are classified as being in the same category as lizards. The first known snake traces back to 130 million years ago. Snakes are almost deaf, they hear through vibrations. They also have no eyelids or legs (obviously). There are 3,000 known types on snakes, within that 3,000 are three families of snakes that are venomous – Colubrids, Elapids and Vipers. The venomous snakes are the snakes that have effects on all haemostasis.

Moore presented 30 different snakes in his presentation. The pictures shown and the information about the snakes are amazing. The first snake he spoke about was the Brazilian Pit Viper or Jararaca, a snake that has heat sensitive detectors that notices change in temperature, is very bad tempered and is medium sized. Lives in Brazil, Paraguay, and Northern Argentina. The Puff Adder is the snake that is responsible for the most human snakebite fatalities in Africa. This snake has long fangs that kill prey through the trauma of the bite, treating a bite leads to amputation. The White-Lipped tree viper is a green snake that is found in India, Thailand, Malaysia, and Indonesia. The Cascabel is the most venomous rattlesnake that is found in Brazil, Bolivia, Paraguay, and Uruguay. All of these snakes, and many more that are venomous, have effects on haemostasis.

Every snake has differences in the way that their venom affects haemostasis. For example, the venom of the Botrocetin snake has effects on the protein domains of collagen A1, and the snake Bitiscentin has effects on the protein domains of collagen A3. Every snake venom also activates platelets differently.

The overall effects that snake venom has on haemostasis are that the protein acts on coagulation factors/inhibitors. Venom also has phospholipase A2 that destroy phospholipids and compete for phospholipid binding sites. Fibrinolytic enzymes also come from venom that is a direct plasminogen activator. Venom has metalloproteinase that degrade the blood vessel extracellular matrix, and ultimately the venom components affect platelets. Platelets are a small disc-shaped cell that is involved in clotting – haemostasis. All of this depends on the snake.

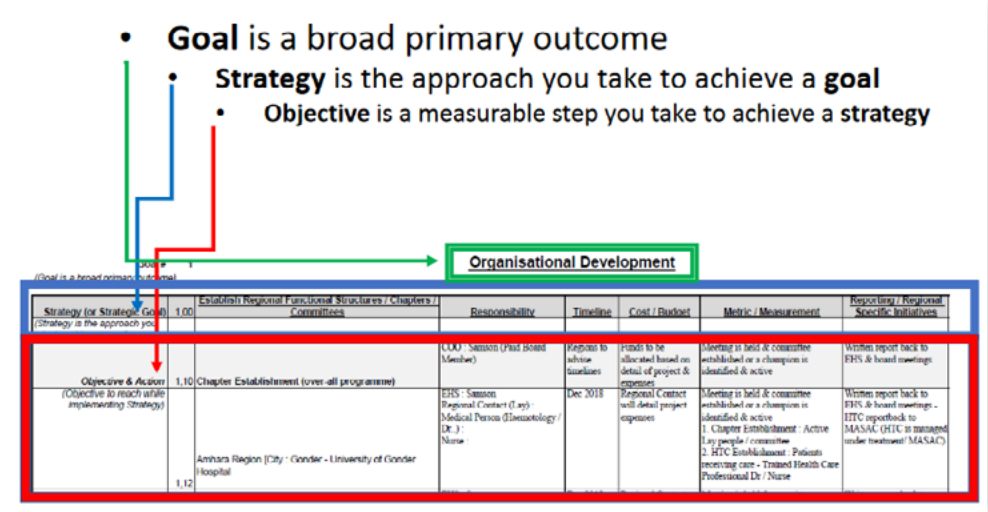
Snake venom is beginning to enter the Pharma world and is used to treat many different cases. The Cascabel powder that comes from the Cascabel snake has been known to be distributed and given out by the 'Snake Man Pete'. This Cascabel powder has cases of curing diabetes and tumours. The venom of Crotavirin, Bothrasperin, Mambin, and Rhodostomin snakes has also been used in cases where cancer has appeared to have been treated successfully.

Moore's presentation of the, 'Effects of snake bites on haemostasis' shows that venom can have a positive effect with haemostasis, impacting on the clotting of blood. Snake venom may be the future of bleeding disorder treatment.

Forward Thinking Patient Organisations

BY KARL ARCHIBALD

Forward-thinking patient organisations was a presentation session done in a different way, with style, with flair, with banter, and with a time constraint that gave each person 30 seconds a slide, or 6 minutes in total, to deliver their message. Moderated by Brian O'Mahony, this engaging presentation style maintained the audience's interest, didn't drag on, and had an appropriate amount of humour to captivate the audience.



Bradley Rayner presented on Twinning Dynamics. He looked at the South African Haemophilia Foundation's transition from being a 'student twin' to a 'teacher twin' with Mauritius. Some interesting facts about Mauritius twinning: between 2012 to 2017 HAM has surpassed SAHF re IU/Capita, they have an office with paid staff, the relationship between HAM and the government is very positive, and they have started a medical twinning with SAHF in 2018.

Bradley highlighted some key requirements for successful twinning:

- The partnerships required similar values
- The lead twin has EXPERIENCE in area requiring development
- Both twins will learn during the process
- Communication with understanding is critical to a successful twinning
- They will seek an understanding do not assume each other's intent
- Culture & religious practices may also impact the twinning.

During the twinning they set short and long term SMART goals, made sure that there were clearly stated primary and secondary outcomes, and that they measured qualitative and quantitative results against their objectives.

Bradley concluded by discussing the impact, and the power, of volunteers within an NGO:

"We ALL have a role as Volunteers to grow our NMO, to unlock further potential, to grow our membership through active participation, innovative ideas, and education. The future then awaits US!"

Evelyn Grimberg was next up, presenting on Blood Relatives: Cycling to Stavanger – Getting on the map: Recognition and fundraising.

Evelyn is very involved in the bleeding disorders community, having been a volunteer with the Dutch haemophilia community for the last 9 years, the chair of the Dutch youth committee, and is currently representing women with bleeding disorders through the European Haemophilia Consortium. Evelyn has a rare bleeding disorder, Glanzmann's thrombasthenia. Through sharing her story, she has become recognised as a strong advocate for women and people with rare bleeding disorders.

As a result, Evelyn was pivotal in the team's success of Cycling to Stavanger for the European Haemophilia Consortium in 2016. The group departed from the University of Utrecht and continued over 1325km to their destination in Stavanger, Norway.

Achieving this feat required extensive forward thinking, as you need with any Big Hairy Audacious Goal. They had to look at the big picture, and then break the process down into smaller tasks. They also needed to obtain coverage to raise awareness, financial support for equipment, and condition their bodies to be able to achieve this undertaking. They undertook extensive training rides, sought expert advice and used conditioning machines at the university.

Once they set off on the ride, they still had to overcome obstacles such as food, route challenges, rest stops, support vehicles, overnight points, and the many punctures along the way. They couldn't have achieved

it without working together, without the input of stakeholders in their community, or without the passion, drive, and planning required to deliver on the result.

With the success of this one, they now have their sights set on doing it all over again.

Engels Rolando Reyes Sanchez spoke next, on planning and initiating projects.

I had the opportunity to work with Engels during his time at SURO in 2011/2012, where he discussed some of the challenges that he had within the Asociacion Nicaraguense De Hemofilia. Today he presented on those challenges, and, now in his role as President, the solutions that he and the board found to overcome those barriers.

He found that the foundation had a glass half-empty approach, and a negative outlook. Also, they did not have experience in big projects, couldn't set a clear objectives, had several health problems, a bad economy, a lack of treatment, and needed more education...

They set out with big aspirations.

1. Advocacy
2. Educate PwH and relatives
3. Volunteer training
4. Work together ANH/SCHQ

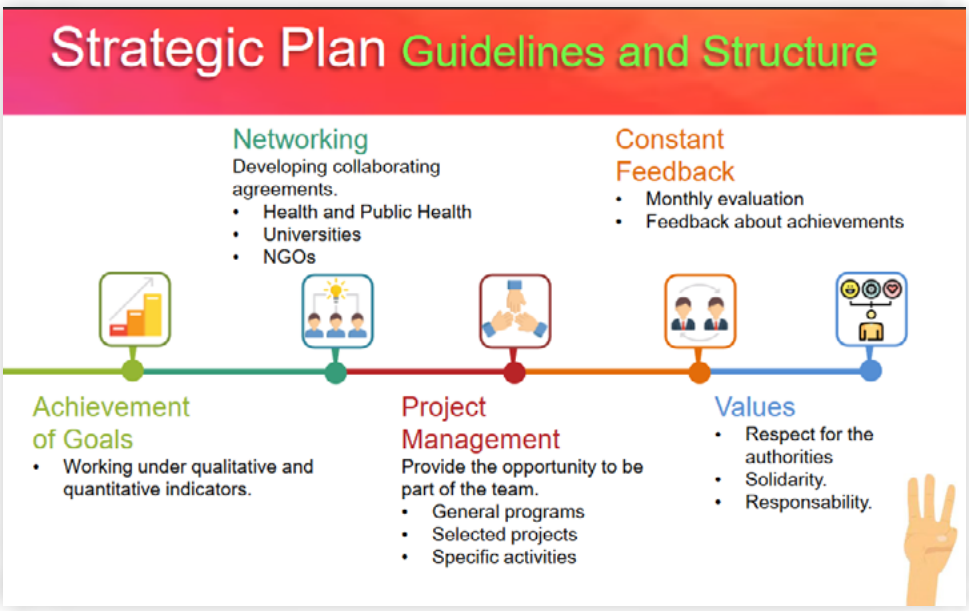
They created a multidisciplinary work team consisting of the board, administrators, counsellors, PR consultant, HR, and Legal counsel, which worked in conjunction with the medical team of haematologists, orthopaedic surgeons, nurses, paediatric doctors, and lab staff.

Together they formulated a campaign that would raise awareness, create structure, hold each other to account, and report back on achievements.

From 2012 to 2017, they worked very hard to achieve their goals. By raising awareness of bleeding disorders and having "diagnosis days," diagnosis grew by 40%.

Here are some of their achievements:

- 8,500,000 IU Factor VIII donated
- 1,755,000 citizens invited to diagnosis
- 315 citizens diagnosed with a bleeding disorder
- 645 People trained including healthcare professionals, medical students, and PwH
- National clinical registry created
- 1 Website : www.hemofilia.org.ni



While they had their barriers, they also had determination and drive. So they started small, elected a board, started a strategic plan for short medium and long term goals, and got to work.

- 113 healthcare consultations
- 75 conferences or workshops
- 129 new volunteers.

Engles concluded that by remaining focused, maintaining time administration, utilising people's experience, playing to

their strengths and skills, continuous development, holding each other to account, self-evaluation, believing in your projects and having a positive attitude, then you are able to achieve your goals.

Next up, our own Deon York, spoke about fundraising and negotiation in today's economy.

If you are unsure who Deon is, he is the chap on the inside cover of this publication under the heading The H Word.

Deon discussed the fantastic fundraising model we all know and appreciate here in NZ, Kiwifirst. He discussed the long-term relationship management process undertaken to raise the funds needed to create a model that is able to sustain HFNZ, enabling us to deliver fantastic programmes and support for our membership.

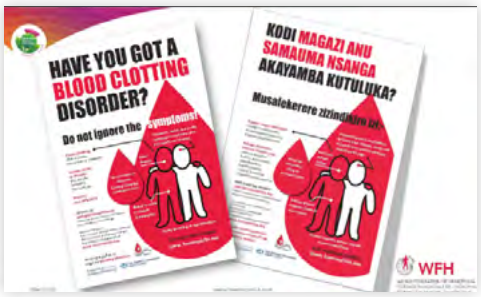
Deon discussed that the Kiwifirst model is unique in the sense that if you attempted to do the same in today's society you may well not get the same result. However, innovation and continuously evolving the model (to a system such as a regular giving programme), enables you to keep ahead of others who are all fighting for the same dollar.

Fundraising is a long-term strategy, just like securing safe and sustainable product supply for the membership. It is down to hard work, a comprehensive database to draw quantifiable information from, and long-term relationships through the NHMG, National Council, Pharmac, the Ministry of Health, DHBs, the bleeding disorder community, and other stakeholders in order to provide a comprehensive care package and the foundation support that we have grown to appreciate.

Susan Warren from Haemophilia Scotland talked next, about a project she undertook when she found herself with a "little bit of free time". She sought the help of Dr Livingstone, and they got to work exploring Malawi, generating a partnership between the two nations.

The aim of their project was to facilitate access to appropriate treatment for people with bleeding disorders in Malawi following a laboratory diagnosis. To get there they had three main goals:

1. INFORM: More people with a potential bleeding disorder in Malawi will be aware of where they can go for diagnosis and treatment.



2. DIAGNOSIS: More people within Malawi with a bleeding disorder will have a laboratory diagnosis of their condition and have that diagnosis recognised on a National Haemophilia Register.

3. TREAT: More people with a bleeding disorder will have specialised Haemophilia care and locally produced treatment.

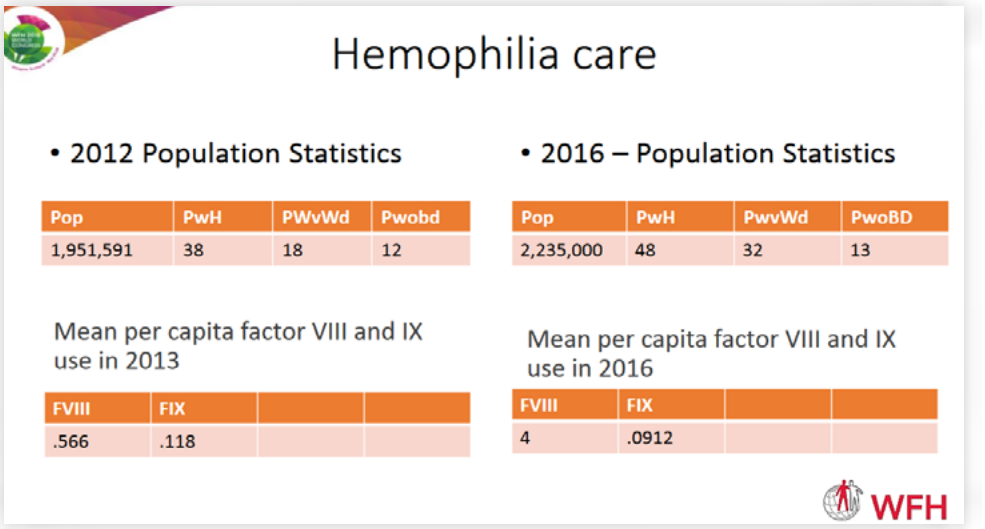
The team understood that it is what we do that makes the change, so there were specific activities they undertook to achieve each goal:

1.INFORM: Initiate a public awareness campaign to identify potential bleeding disorder patients. This includes newspaper and radio advertisements, posters in hospitals, social media, and press conferences.

2. DIAGNOSE: Once potential patients come forward they will be referred correctly as a result of training delivered in central and regional hospitals. A new haemophilia clinic will have access to laboratory diagnosis tests using reagents supplied by the project. Records of diagnosed patients will be held on a new national haemophilia database in Malawi.

3. TREAT: Well-qualified haematologists will treat all diagnosed patients, including at the new haemophilia clinic. They will be treated with locally available therapies which are currently under used.

Throughout this project, which they undertook independent of the WFH twinning programme, they also raised money through various campaigns to fund the programme in Malawi.



Susan said that because both parties were so eager to get involved with the programme, what they did really made a difference.

Last up was **Ahmed Bahey**, who talked about the transformation of haemophilia care in Qatar.

Ahmed is the General Secretary for the Hemophilia Society of Qatar.

Qatar has a population of 2.5 million with an annual healthcare investment of $5.7b. Haemophilia care in Qatar is provided through two main hospitals; they have had Prophylaxis since 2014 for FVIII recombinant, and in 2016 adopted electronic health records.

Ahmed discussed the comparisons between haemophilia care in 2012 and 2016, and the positive impacts seen during this time.

This outcome has been because of a hard working multidisciplinary team, who have grown awareness and education by hosting self-infusion workshops, developing and utilising expert nurses and haematologists, developing an orthopaedic programme, physiotherapy classes, patient education days, and dental hygiene seminars, and via ongoing media coverage through TV interviews.

In 2017 Ahmed received the Stars of Excellence award from the Minister of Heath for his work in transformational Haemophilia care.

These speakers, and the conference as a whole, provided a great understanding and insight of what people are doing within the Haemophilia community around the world. There was such a positive outlook about the advances in therapies.

On a slightly bias note, throughout the conference, people mentioned that comparatively, NZ has really lead, and in many areas is still leading, the way for innovation and change when it comes to bleeding disorders care. NZ is highly respected, and was well represented at congress, and I think that is worth a pat on the back.

Gaining insight into the complexity of pain in patients with haemophilia

BY LYNNE CAMPBELL

Speaker: **Nathalie Roussel**, Assistant Professor at the Faculty of Medicine and Health Sciences University of Antwerp, Belgium.

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

Pain is subjective and multi-dimensional.

Natalie Roussel has a background in Physiotherapy. She outlined the physiology of normal pain and pain pathology. This was then compared with abnormal pain, where in a chronic condition such as haemophilia pain hypersensitivity results. Constant and widespread pain contributing to a reduced quality of life is prevalent in many with haemophilia, yet very few studies have examined the (patho)physiology of pain in PwH.

Other conditions such as osteoarthritis and low back pain have provided an insight into the role of the central nervous system and the brain in the cause/origin and/or maintenance of pain where chronic pain results. Studies in this area have shown that pain does not equate to damage. Pain is detected in the brain; however, there are many, many peripheral nerve centres that detect a sensation that is then interpreted by the brain as pain. This has proved invaluable in assessing and managing pain.

PwH are living longer but many are now living with constant pain. In those with severe haemophilia, bleeding causes acute pain and arthropathy causes chronic pain. There is no consensus in pain management, and different treatment centres treat and manage pain differently.

In a person with haemophilia, the first priority is always to exclude bleeding as a cause of pain.

Next, the primary mechanism contributing to the pain has to be determined (i.e. nociceptive, neuropathic pain or altered central pain processing). The type of treatment required to address the pain will be different depending on the underlying pain mechanism.

With better insights into pain mechanisms, it may be possible to establish a specific treatment plan:

- In the case of nociceptive pain - Medication in PwH?
- In the case of neuropathic pain - Tricyclic antidepressants
- In the case of neuroplastic pain - Pain is not always a reliable signal in deciding whether medication is required.

The point was made that, in assessing pain, consideration needs to be given to both the physiological and psychological elements contributing to the expression of the pain. Patients frequently experience negative feedback when seeking relief from pain.

Because of the neuroplasticity of the brain, cognitive emotional desensitisation can be beneficial. Stress, depression, worrying, fear of movement, and hypervigilance all contribute to cognitive emotional sensitisation.

People with haemophilia experience pain in expected ways, such as joint pain, however pain is also felt throughout the body, which suggests widespread pain hypersensitivity.

The patient has an idea as to what is causing pain. Experience can lead to accompanying fear and anxiety that certain actions or activities will result in pain. This can in turn lead to an abnormal pain physiology where brain activity increases in an altered central pathway, which leads to a change in pain thresholds, hypersensitivity, and heightened brain hyperactivity.

Pain pressure thresholds (PPTs) can be evaluated clinically. Roussel believes that, for the effective management of pain, clinicians need to explain the neuropathic pathway for pain to patients who suffer from altered neuro sensitivity. Research focus is now on ways of decreasing pain thresholds and reducing temporal stimulation in the brain's altered pain mechanism in PwH. Other studies have shown that pain thresholds decrease with exercise although there have not been a lot of studies in PwH. Activation of other parts of the brain have been shown to be effective in distracting away pain from peripheral receptors feeding into the pain neuromatrix of the brain.

Gene Therapy

BY ROSALIE REIRI

Gene Therapy

A cure for haemophilia is within reach through gene therapy. This silver-bullet has made a profound impact on treating haemophilia in recent years, since as early as 2015. Gene therapy has been coined as a disruptive technology that is out-shining the evolution of conventional treatment over the last fifty years. From the sixties plasma derived products, the nineties move to recombinant clotting factors, to 2014 extended half-life, to this next revolution in treatment. What makes gene therapy the ultimate solution over other approaches is the full coverage one receives with no need to have prophylaxis, on demand, sometimes, or anytime. Scott Gottlieb stated in a press statement in 2017 that "I believe gene therapy will eventually become a mainstay in treating, and maybe curing, many of our most devastating and intractable illnesses".

What is Gene Therapy?

A simple explanation for gene therapy is that scientists have been trying anything and everything to push stuff into cells. By and large, by what is published, nothing has worked other than the insertion of a virus, or in other words gene therapy uses a genetically engineered virus as the vehicle to replace the missing stuff in the cell. The AAV virus historically goes as far back as 25-45 million years ago, found in primate animals. The knowledge base has been around for fifty years with extensive works from Donald Ronsfield. Within the same timeframe, it has been found that the virus can be introduced into the cell intravenously. The virus needs to get into the nucleus, which then it transports the desired gene into the centre of the cell.

How do we know it works?

We know gene therapy works as it has been proven in animals, in particular mice, as well as in 2300 human trials. Although we know it works, we don't know exactly how it works. The success of the trials have been measured by the frequency of bleeds, and by comparing data pre gene therapy to post gene therapy. Of the clinical trials

shared, all results have been outstanding. The game changer showed results in patients who had large number of bleeds before starting gene therapy, with up to 100 infusions a year, where, the following year, after receiving treatment, bleeds decreased to the point of having no infusions whatsoever. The risks from all trials to date have been minimal.

Gene Therapy and the Future

Gene therapy, gene editing, and cell therapy are all long-term solutions for haemophilia. Each approach has different implications for efficacy, safety, and success. Gene editing corrects the faulty gene by inserting the correct gene in its place whereas cell therapy transplants whole cells into a person. Cell therapy has been topical in the news but it is not ready yet.

So who is eligible for gene therapy? PWH Factor VIII, IX and those with an inhibitor. It is not clear now if children can be treated, as we know that in animals their bodies kicked the DNA out. This poses the question; at what age can children be treated?

Although gene therapy can help PWH, it doesn't get rid of the gene entirely as we know that the haemophilia gene will still be passed down to all daughters. Lastly, we know that 25 % of PWH in the world is treated, 75% still do not have access to treatment, which shows a huge disparity to those who have access and those that do not.

In conclusion, the light at the end of the tunnel for treatment and haemophilia is bright. The advancements of technology and medical science need to be celebrated. Although the impact of gene therapy for haemophilia is astounding, it is currently still in its infancy. Therefore the need for continued research to explore all the unknown areas of gene therapy in order to have a critical understanding of the benefits and pitfalls.

In closing, I found this particular session fascinating and I appreciate Glenn Pierce who gave a clear keynote with visuals and explanations, which helped me understand, as sometimes I find medical sessions hard to comprehend. The conference as a whole was amazing. I never get tired of new ideas and new research but my favourite is hearing people share their personal stories. Whether they are the patient, caregiver, doctor or the psychologist. I found all the sessions I attended the speakers were genuine, and spoke from a place that really touched me. I felt as if they held a view that was holistic and wider than just talking about a patient with a diagnosis. This experience was enriched by attending with others from NZ. It made the time fun, we were able to reflect on a day-to-day basis as a group and stay connected throughout the entire conference.

Haemophilia Gene Therapy: From trailblazer to game changer

BY LYNNE CAMPBELL

Speaker: **Thierry van den Driessche** - Professor, MD, and Director of the Department of Gene Therapy and Regenerative Medicine at the Free University of Brussels

Gene therapy has proven long-term efficacy and safety in many other inherited disorders and in some cancers. In addition, regulatory authorities for other genetic diseases and complex disorders have already approved several gene therapy products.

Gene therapy is an experimental technique that uses genes from a vector to treat or prevent disease by replacing missing or malfunctioning genes. In the future, this technique may allow doctors to treat a disorder by inserting a gene into a patient's cells instead of using drugs or surgery.

More recently preclinical studies have proven successful in haemophilic mice and dogs, and since the 1900s, great progress has been made in gene therapy as an intervention for blood clotting defects in haemophilia. Initial successes were recorded for Factor IX deficiency, where the focus was centred on liver cells. Since then, greater progress has been made in Factor VIII deficiency, where the intra-cellular biochemistry involved in getting the vector to target and then survive and function within the cellular matrix is more complicated. Finding the right molecular key to enter the target cells and survive the body's immune response is the primary consideration. It is imperative that the AAV in the liver cells survives the immune response.

The efficacy of Adeno associated viral vectors (AAV) has now been established in human trials. It is important to note that a viral vector is not a virus; therefore, there is no chance of viral infection. In paediatric patients, the liver cells are still dividing, so it is more difficult to get stable genome integration.

For inhibitors where there are pre-existing antibodies, because of the unique nature of the liver, gene therapy could potentially eliminate the pre-existing antibodies.

Estimates from trials suggest intervention from gene therapy is effective for seven years.

Gene therapy is not a magic bullet but provides an optimal therapeutic window. Although it is promising, several therapeutic challenges remain. The situation in children and in those with inhibitors is unresolved as is the situation

for those where there is a pre-existing immunity to the adenovirus-associated viral vector.

Progress is such that now sustained therapeutic VIII and IX expression levels are being achieved in people with severe A and B. Barriers such as interpatient variation in factor expression, sustained response, safety, and risk of inhibitor formation remain considerations.

Healthy Ageing

BY TUATAHI PENE

This session was presented by seven speakers, and chaired by **Nicholas Goddard.**

Dr Alison Dougall spoke on oral health in PwBD, and how it can be difficult to manage later on in life. The specialty she is concerned with is the diagnosis and study of the causes and effects of diseases affecting the mouth area.

One of the biggest misconceptions around treating PwBD with bleeding gums is that the patient often thinks that gum blood is a bleed that warrants factor treatment, when the bleeding can merely be a symptom of inflamed gums. Other problems of the mouth can be misdiagnosed by patients because of poor information and, in some cases, the unhelpful attitude of "I'll go only when it hurts."

Educating patients to understand what happens in their mouths is crucial for living longer and better lives.

The second speaker, Dr Angela Forsyth, gave her opinion on aging from a physiotherapist point of view.

The impact on age-related musculoskeletal patients differs drastically from people living with haemophilia compared to those living without. The accumulated damage of bleeds results in symptoms presenting as weakening of synovitis, soft tissue damage, muscle wasting, and many more as people age. There is a high risk of structural and functional failure of joints, erosion of articular cartilage, and alteration of subchondral bone.

The best course of treatment involves exercise to help maintain (not increase) bone density in adults. Impact training may be more limited due to the person's history of bleeds, but muscle strengthening can promote ease of body functions and movements.

Nurse Cathy Harrison stood as the third speaker, talking about healthy ageing from a nurse's perspective.

With patients living longer, the field of ageing PwBD is now becoming a new area of study. Nurse Cathy touched on important topics to focus on from childhood all the way through to old age:

- The importance of good nutrition
- Physical activity
- Maintaining relationship
- Sleep
- Mental health for care
- Maintaining social networks

Concentrating on these areas can help maintain better quality of life for PwBD, and promote a longer life with no bleeds.

Ruud Bos is a social worker in the Netherlands, who spoke on healthy ageing from working mainly with senior clients.

Ruud brings a unique perspective, gained from occupying a lot of his time with seniors. He has found that social interactions with their peers have helped their wellbeing, from small outings, games, and activities, their activities result in greater happiness.

The downside of his study is that long-term sufferers of chronic pain related to a history of bleeds have shown some adverse effects on mood and mental health issues; some fearing a loss of independence and having to rely on others.

Similar to Nurse Cathy, Ruud relies on the bullet points above to help treat seniors in their times of need.

The fifth speaker, Dr Margaret Ozelo, had spoken about her perspective as a Haematologist on the Healthy ageing subject.

A comparison study of Haemophilia A patients living in the United States of America shows a 2% increase of PwBD living over the age of 65 from the year 2011 through to 2015. The results shows that PwBD are living longer lives, which is fantastic, but the question remains, how well are they living?

Dr Margaret's study shows that PwBD living over the age of 65 are suffering from additional ailments, when compared to the average person. Haemorrhagic complications continue to be the leading cause of death in haemophilia. Among patients infected with HIV and/or HCV, the causes of death are associated with related complications, including cancer.

At present, there is limited evidence and experience in managing these conditions. Given that the population is aging slowly, it may take some time to generate high-quality data.

Carlos Safadi was the sixth speaker, on the ageing population of people with

haemophilia living in Argentina. Carlos spoke about how in the mid-1940s the Haemophilia community came together to help support one another. This organisation later progressed to become the world federation that we know of today.

One of the hardships that the ageing population is facing now is the lack of government involvement in long-term health policies, where the view on cost is about spending rather than investment in people. The difficulty with this is that patients who are suffering long-term illnesses are concerned about receiving proper care, the stresses brought about by no assurances of treatment, treatment centres, or what the future will look like for them.

The final speaker, Randall Curtis, spoke about the ageing population in the United States of America living with Haemophilia.

Similar to the situation in Argentina, the lack of Government support is a big issue that people with haemophilia are facing in the US. There, funding is allocated by state, and the state dictates how it should be spent. The only other avenue is relying on insurance companies and the limited policies that are available to patients with long-term illnesses.

With the rise of PwBD living longer, it is apparent that there are limited facilities available to treat the ageing populous. Lack of funding means lack of care, and a lack of care results in people suffering unnecessarily with their illnesses.

HFNZ Women's Experience of Menstruation poster report

MĀHIA NIGHTINGALE-PENE

This report is about the success of the poster I presented, 'HFNZ Women's Experience of Menstruation'. Our Comms Manager, Phil Constable, put this poster together. You might remember this topic from the article in the March 2018 Bloodline. All of the korero from that article turned into a poster. At the World Federation of Haemophilia 2018 World Congress, the poster had a lot of attention and prompted further discussions about women's experience of menstruation, which I thought was a success.

On the third day of the conference, I was scheduled to present this poster in three different slots throughout the day. Next to me was a woman from America presenting her poster about menstruation products, across from her was Karl presenting his poster about HFNZ Youth.

At every session, people who were intrigued approached me. There were two main reasons why I think the poster was such a success. One reason was Phil's great creative work. The poster was so easy to read. In comparison to other posters in the same area - there were fewer words, the main points were enlarged to catch attention, and the entire poster was straight to the point. Phil did a great job at making the poster easy to read and eye catching.

The other reason I think the poster was successful was the content. It was obvious that the content was a success because people were commenting and asking questions. The comments received were all sympathetic comments and people were amazed at the results. The results that people were most surprised by were the fact that 82% used more than one sanitary product at a time and 60% need to change their sanitary items at least every 2 hours at peak times. People who were interested were also disheartened to read that some women pass clots the same size as a tampon. One person commented on not being able to imagine this because of how much pressure there already is in the world, she was very sympathetic saying that she hopes the submission to Pharmac is a success.

Questions asked were those associated with what can we do to help these women, what are the next steps, and why are costs so high in NZ. With the help of having the March 2018 Bloodline handy and having read it 5 times I was able to answer all questions. They were happy knowing that the survey and results that are on the poster are being sent to Pharmac.

The majority of people also offered solutions. The solutions were the suggestion of the sustainable and reusable approach – like menstrual cups and a period-proof garment

HFNZ Women's Experiences of Menstruation

By Phil Constable. Presented By Mahia Nightingale-Pene

Early in 2017, one of our members asked whether there were subsidies available to help reduce the high cost of sanitary items for women with bleeding disorders, or who carry the haemophilia gene. This led to a wider investigation into the ways that HFNZ women experience menstruation, the effects on them, and on their families/whānau.

What We Did

We conducted a survey of affected women from the HFNZ database. To inform the survey, we accessed related research, and formed an advisory panel of HFNZ women. We emailed the resulting survey to 229 women.

From that group we had 71 responses, a 31% response rate, and a very good sample of our people. We collected both quantitative and qualitative data.

87%

self-reported excessive bleeding, menorrhagia, during their period

46%

reported financial hardship related to the use of sanitary products

"You sometimes feel you are living half a life. Iron deficiency following heavy bleeding leaves you feeling tired, grumpy, and unable to cope."

What We Found

The results were, in some cases, astonishing!

Of the 71 women who responded, 87% indicated that they had experienced excessive uterine bleeding, characterised as menorrhagia, and 64% indicated that their menorrhagia had continued for more than three years.

Most of these women recorded having to use more than one sanitary item at a time. Some women needed to change their sanitary product(s) as often as every 10-30 minutes, and were passing clots big enough to push tampons out, resulting in leakage and damage to garments, bedding, and furniture. Furthermore, 62% of respondents recorded menstrual periods regularly lasting in excess of 8 days per month. The frequency of use, and quantity required, led to significant financial hardship for 46% of women, and their families/whānau.

77% of respondents reported having undertaken treatment specifically for their menorrhagia, including the contraceptive pill, Mirena, tranexamic acid, uterine ablation, and hysterectomy.

82%

used more than one sanitary product at a time

62%

had periods lasting longer than eight days each month

77%

had undertaken treatment specifically for their menorrhagia

60%

need to change their sanitary items at least every 2 hours at peak times

"When we were a young family this cost for products prevented us from buying other essential Items"

The Unavoidable Truth

It is clear that women with bleeding disorders, or who carry the haemophilia gene, are inordinately affected by extreme symptoms, and by the very high costs associated with menstruation.

Haemophilia Foundation of New Zealand Inc. HFNZ

called the Thinx. The menstrual cup I am very aware of, and absolutely agreed with. The Thinx period-proof garment on the other hand I had not heard of yet. The lovely woman who told me about this product sent me a link to the products, and explained the product to me. The garment is underwear that has four layered patented technology for ultimate period protection. The garment can be used to replace menstrual products or as extra protection.

Well done to those who put the survey together, worked out the findings, and worked on the presentation of the poster. It was my pleasure to present the poster about HFNZ Women's Experience of Menstruation at the World Federation of Haemophilia 2018 World Congress. It was well worth my time, I had the opportunity to talk to so many people who were so interested in the poster, and I learnt myself about different menstrual products and felt the sincere sympathy from others for those who go through the struggle of having heavy menstrual cycles.

Impact of Hemophilia on relationships

BY MAHIA NIGHTINGALE-PENE

This session had three different speakers presenting on the following topics:

- Impact of hemophilia B on relationships - Susan Cutter
- Mothers' relationships with their sons with hemophilia – Erin Stang
- The value of friendships through the lifespan – Linda Dockrill

Impact of hemophilia B on relationships

Susan Cutter, LCSW, MSW, MPA

Susan Cutter is the Assistant Director for the Peen Comprehensive Hemophilia and Thrombosis Centre at The University of Pennsylvania Medical Center in Philadelphia, USA. She is also the social worker for the centre and has nineteen years of experience in hemophilia.

Cutter begins by identifying the impact of hemophilia B on family relationships, such as how illness in one family member places physical and emotional well-being of all family members at risk. She also talks about the stretching of a family's resources to focus on an individual with chronic illness and how the stresses can change the family structures and relationships between the parents. Despite this, she shows how remarkably resilient families are at adapting when a child in the family has a chronic illness.

A survey focused on the impact of hemophilia B on relationships with partners, family, peers, and colleagues. This survey included 299 people with Hemophilia B and 150 parents/caregivers of children with Hemophilia B. From the adult respondents 54% were either married or in a long-term relationship. 87% felt that their hemophilia impairs their ability to develop close relationships with their partners, both past and prospective. Primary reasons included their worries about being able to provide a family in the future (45%), the impact of their hemophilia in future years (56%) and the reaction they may receive when disclosing their hemophilia diagnosis (42%).

Perceptions of Vulnerability, Protective Behaviour, and Stress in Mother of Sons with hemophilia

Erin Stang, LCSW

Erin Stang is a licensed clinical social worker with fifteen years of social work experience caring for individuals and families affected by bleeding and clotting disorders. Ms. Stang is the Social Worker at The Hemophilia Center at Oregon Health and Science University.

The objective of Ms. Stang's research was to compare mothers of affected sons who have a known family history of hemophilia to those with no known family history of hemophilia. It identified perceptions of vulnerability, maternal protective behaviours and reporting parental stress in mother, son and the relationship.

The study included 74 eligible mothers. Twenty-nine mothers self-reported positive carrier status (74.3%). Twenty-one mothers had a known family history (52.8%). Four mothers of two sons with hemophilia (10%). Thirteen mothers had single children, and 37 years is the average age of these participants. The results of this study demonstrated a complexity of perceptions and behaviour exhibited by all mothers with sons with hemophilia.

Points of discussion were that further research needs to be initiated to explore the complex psychosocial differences in individual mothers of sons with hemophilia behaviour and perceptions and how it relates to family history. Further research needs to be initiated to explore behaviours and perceptions of mothers of only children.

The value of friendship through the Lifespan "You can count on me"

Linda Dockrill, Registered Social Worker, NZ

Linda Dockrill is a registered social worker who works as practice leader in a Non-profit organisation supporting

children and families with parenting in Christchurch. Linda was previously employed by the Haemophilia Foundation of New Zealand.

This presentation identified challenges and strengths people with bleeding disorders encounter in friendships. It also explored cultural, gender, age differences and friendships across the lifespan of a person with a bleeding disorder.

These were broken down into six categories ranging from infancy and early childhood to older adulthood. For infancy and early childhood (Birth - 6 years) friendship is established firstly amongst family, and then builds on attachment from family relationships. True friendship can then be seen for 3-4 year olds and is used to describe playmates. In early and middle childhood (6-12 years) children begin to develop their own views, and identity can be different to others. School may be the first time they are away from the care of immediate family members and this develops lifelong friendships. Adolescent (12-20 years) friendships are part of a larger network of relationships. Friendships become central to life and moving away from parental influences occurs. Peer education groups and social media influences are also other forms of support during this period. In Early Adulthood (20-40 years) bleeding disorders are integrated into identity, joining the workforce, partnerships, and parenthood. In Middle Adulthood (40-65 years) workplace friendships and intimate family reltionships are key. Interest in connecting with other people with bleeding disorders grows again. In Older Adulthood (65+ years) people with haemophilia are often heavily dependant on partners, social supports, and friendships become a buffer in coping with illness and increase wellbeing.

There are certain recommendations for health professionals, which include promoting and protecting positive family relationships, utilising referrals to mental health specialists, and promoting the oppotunity to develop friendships through membership organisations, chapter activities, camps, and workshops.

Joint Disease

BY TUATAHI PENE

Dr Steve Pipe chaired this session on joint disease, an analytical discussion with three key speakers presenting new insights into therapies for patients.

Dr Lize van Vulpen spoke on new findings in pathogenesis in blood-induced joint damage leading to joint disease. Bleeds in joints are the leading cause of joint disease in people with haemophilia. Since introducing prophylaxis to PWBD, studies have shown no reduction in joint damage, and further possible onset of Rheumatoid arthritis.

The pathogenesis, or development, of joint disease seems to be multifactorial, where several bleeds in the same area will cause chronic swelling, deformity, and a possible progression in forming tumours. Dr Vulpen has developed a study that demonstrates the biochemical markers of joint tissue damage, and the damage increase shortly after a single joint bleed with established haemophilic arthropathy joint damage. She hopes that the use of biomarkers will one day be useful in monitoring the impact of a joint bleed, and in providing an evaluation of treatment for such bleeds. The aim is to create a diagnostic tool to identify and assess the injury, in order to deliver the best course of treatment.

Dr Katharina Holstein presented on the topic of medical management of pain and function in joints. Bleeds in joints create a

vicious cycle of bleeding to the synovitis areas, which can lead to further bleeds into the bone region. With weakened joints, the possibility of bleeds to the joint area increases, and the cycle starts again. Pain is present in every step of the cycle from the initial bleed to the weakening of cartilage and bone.

The average person, when dealing with pain, will alleviate the painful area by shifting weight to another area of the body to compensate. The result of this causes increase joint loads in those areas and may result in bleeds in the joint.

General principles of pain management in haemophilia involve the following:

- Factor replacement
    - To treat acute bleeds
    - To prevent bleeds, chronic synovitis, and arthropathy
- Pharmacological treatment
- Non-opioids, non-selective and Cox-2-selective NSAIDs
- Weak and strong opioids
- Adjunct medications
- Physiotherapy
- Psychological support
- "Causal" treatment (like surgery, radiosynoviorthesis, anti-inflammatory treatment etc.)

The pharmaceutical treatment of patients via drugs that dull the pain receptors can sometimes lead to dependency. To lower the risk of dependency it is important to identify the pain in relation to the bleed, and manage the best course of treatment.

Dr Holstein explained how the brain works in identifying pain; explaining the different receptors that send signals to the area in the brain letting you know the body hurts. When Panadol is taken, the pathways get inhibited by the drug and trick the brain into thinking it is okay.

Using drugs moderately to provide ease of pain can be useful to aid patients and their recovery period of the bleed, to further help with therapy exercises.

A multidisciplinary treatment of haemophilia-related chronic pain is needed; early physiotherapy may help to prevent chronicity in joints. Education about pain neurophysiology should start early in patients, to help give comprehensive self-assessments, and to help health care professionals make the patients aware the risks of taking pain medication. The option of pharmacological therapy should also be available to the individual.

The third and last speaker, Dr Carlo Martinoli, spoke on the role of imaging in the assessment and management of haemophilic arthropathy.

A condition characterised by repetitive hemarthrosis, and ultimately joint deformation, in patients with bleeding disorders is what defines haemophilic arthropathy. It can occur when there is one or few bleeds in the joint.

Diagnostic imaging has the potential to reveal damage of said joint. Ultrasound imaging of the joint provides the health care practitioner an internal view of the area. From the imaging, the practitioner can assess the damage done by the bleed and respond with the appropriate treatment and care.

A further joint healthcare assessment is a key step made by clinical physicians by including diagnostic imaging, like X-Ray, MRI, and Diagnostic Ultrasound. A bleeding history of the joint/muscle under assessment, patient home-infusion records, and patient registries are also crucial to maintain accurate data.

Creating a protocol beneficial for patients will entail a combination of assessment strategies and procedures to take the most accurate and reliable results for the clinical assessment of joint and muscle bleeding, while still being feasible in a clinic setting.

Patients informing policy decisions

BY TUATAHI PENE

Karen Facey presented on how best patients and their caregivers can influence policy decisions. Deon York chaired the presentation.

Patients provide a unique perspective about living with their disability, that can be very informative to any health service provider. Their experiences of treatment regimes, and the development of their care, can be used as references to inform policy makers. Scotland's health services have involved patients to provide input in policy making for many years, the outcomes of this partnership have resulted in many positive cases, but also some conflicting outcomes, that have the potential to sway decision-making.

When forming a committee of people who have a single goal, it is important to have a procedure in place to find out whether the evidence submitted is relevant or not. Karen spoke of a number of different relationships that can be of use for such procedures, such as:

- Evidence-based practice: where a shared triangle relationship exists between the patient values, clinical expertise, and international evidence (external research).
- Evidence informs policy: where there is a shared relationship between the patient perspectives and experiences, the local experts, and the best available internal evidence.
- Health Technology Assessment: where there is a relationship that has a range of national multidisciplinary expertise and international evidence (external research), but there is no patient involvement.

The findings from the above procedures show that forming policies purely from an analytical point of view (i.e. no patients) provides a very limited opinion, that in some areas were detrimental to the people they were aiming to help. Procedures that involve interactions with patients also provide mixed results, but have far higher quality of outcomes, and demonstrated a range of knowledge that even health professionals were unaware of.

Healthcare professionals, when involving patients in policymaking, need to accept the different kinds of knowledge provided. A collaboration of different perspectives can improve service design and outcomes; such participatory processes are complex, and evolutionary to the procedures, but can be beneficial.

There is an ethical viewpoint that patients can be influenced by external information that may cloud their understanding of what they are saying, which in turn can affect research and policies. To limit such occurrences, when forming a committee of relationships, it is best to identify the right people, bring them into the process as early as possible, and provide training in the role that they will be filling.

Structuring objectives, and knowing the people who you are working with, is the first step to ensuring great success in policy making. Patients provide a wide range of experiences and unique insights that can contribute to creating procedures that best suit their needs. It is also important to recognise that there are different skills required for different approaches – summative or interpretive, knowledge or decision support. The development of transparent, formalised methods for policymaking has enabled committees from a variety of backgrounds (clinical/non-clinical, research/informatics) to be successful.

The Psychosocial Day: Keeping the Energy Going

BY NICKY HOLLINGS

"The vision of the WFH Psychosocial Committee is to improve quality of care for people with bleeding disorders and to increase their quality of life by making psychosocial care an integral part of a multidisciplinary approach to health care."

Richa Mohan started with an update of WFH committee work, and what the Psychosocial Committee have been achieving since the last congress:

- Piloting the Curriculum: WFH Regional Psychosocial Workshop - Belgrade, Serbia, 4-5 December 2016
- National Psychosocial Workshop - Istanbul, Turkey, 7-8 December 2016
- Youth Leadership Workshop - Panama City, July 2017
- Psychosocial Training - Cairo November 2017

Music and Mental health of PwH - Tim Ringgold

Tim was kind enough to share his personal experience of loss and grief at losing his daughter to a genetic disorder. As he and his wife went through the devastating process of her dying, they used music to help with her distress. When she was in pain, they put music on that helped slow her breathing, so she was able to stop crying, and Tim and his wife were able to enjoy their time with her.

Tim discussed the impact of music on a person, how every person has had a story that is connected through music. Music releases dopamine, "the feel good hormone." The brain areas involved in music are also active in processing language, auditory perception, attention, memory, executive control, and motor control. Using music therapeutically can influence people's mental and emotional health. When people are in times of distress, putting on music that emotionally connects to a time when they have felt safe and loved may alleviate the distress, it can help lift mood, and it can used to reduce anger and aggression. It can also help with pain management, through lessening muscle tension to help with relaxation, rhythmic breathing, and restfulness.

Dave Grohl "that's one of the great things about music. You can sing a song to 85,000 people and they'll sing it back for 85,000 different reasons".

Mindfulness - Georgia Panopoulous

Mindfulness is a practice that originated out of Eastern meditation; it is 'paying attention on purpose, being present in the moment, without judgment of thoughts, feelings. A practice of being present'. Practicing mindfulness takes patience and practice, it teaches us to respond rather than react.

MRI scans show that after an eight-week course of mindfulness practice, the brains fight or flight centre, the amygdala, appears to shrink. This primal region of the brain, associated with fear and emotion, is involved in the initiation of the body's response to stress. Mindfulness practice produces a measurable reduction in the markers of stress and inflammation.

Managing pain with mindfulness isn't about blocking the experience, it is refraining from engaging in the processes that makes it painful, it is a change in perception.

Mindfulness benefits are a reduction in rumination, stress, emotional reactivity, blood pressure, depression, anxiety, pain, and insomnia. It also contributes to an improvement in immune response, focus, concentration, memory, cognitive flexibility, relationship satisfaction and quality of life.

Art therapy - N Taheri

Art therapy is a form of psychotherapy involving the encouragement of free self-expression through painting, drawing, sculpture, dance, and music to improve a person's physical, mental, and emotional wellbeing.

Art therapy helps with personal integration where people are able to bring conflicting emotions together in one coherent image, which is often difficult to do with words alone. Art expression offers an experiential space where individuals can respond to difficult feelings in a new way. This can increase a sense of self confidence and self-efficacy.

The beauty of art therapy is that it is available to anyone of any age. It is a way of communicating. It can be a useful tool for people who struggle to express themselves through words.

Art therapy can give the person an ability to express their feelings through any form of art. Arts are a powerful expression of emotions and can help relieve a lot of stress, anger, and sadness.

PEP without boundaries: A multi country programme

The Parents Empowering Parents (PEP) Programme: A programme of Energizing and Empowering Minds.

PEP offers a psychosocial intervention programme proven to empower parents having children with bleeding disorders around the globe.

Take-aways:

- PEP is culturally adaptable
- This programme has been changing the lives of parents of children with bleeding disorders around the world since 1996, even when little if any medical treatment is available
- The PEP1 programme has now been revised, updated, and refreshed
- The original PEP1 programme has now been translated in to Spanish
- The PEP2 programme, for parents of adolescents ages 13-19, will be released in fall of 2017.

PEP2 programme was developed to pick up where PEP1 leaves off, to address the effect of bleeding disorders on adolescent transition. The programme aims to help families, both parent and teen, to gain insights and open doors to clear communication, gaining understanding of each other.

It teaches parents skills to empower their teens to become independent, capable, confident, productive adults.

YOU LEAD Programmes: Training Programmes for Youth Leaders in Haemophilia – Richa Mohan

India has 80 active chapters; youth have had a group in every chapter since 2008. Leaders have to be trained with leadership concepts and practices that help develop them as leaders for themselves and their community.

The programmes goals are to support youth to face the challenges of adolescence to achieve his or her full potential to be able to become a leader. Personal competencies are developed through social, ethical, emotional, physical, and cognitive abilities that have been grown through activities and experiences.

The youth leadership programme teaches youth self-awareness, to empower themselves to get the best of themselves.

Five workshops were held with 15 sessions of 3 hours duration. Three hundred youth were reached via this process. They were encouraged to express their views and ideas. and to learn how to take on responsibilities for their chapter, including:

- Administration
- Medical management
- Communication
- Data management

- Resource mobilisation
- Finances
- Projects
- Networking
- Government lobbying.

WF Leadership Programme - Antonio Jose Almeida

The purpose of the programme is to empower the next generation of leaders in the bleeding disorders community, and ensure that they are well prepared to take over leadership roles. To ensure the future of the WFH it is important to continue empowering youth, and building their capacity.

Programmes that fall under this umbrella include:

- WFH youth Fellowship Programme/ Susan Skinner Memorial Fund
- Advocacy Programme
- Hot and new Youth Twinning
- Global NMO Training/NMO skills Training
- WFH also produce a range of resources for young people.

Outcomes and Impacts:

- Help youth leaders understand the importance of their contribution to the BD community
- Develop the innovative spirit of youth and enable them to build support for their patient organisation
- Empower and enhance the skills of future youth leaders
- Provide a space to facilitate (in person and virtually) interconnections, communications, knowledge and experience sharing.

Relationships and self esteem

BY NICKY HOLLINGS

This very interesting session was presented by:

- Lisa Thomas, a relationship therapist who works with couples
- Greig Blamey, a physiotherapist who is currently on the musculoskeletal Committee of the WFH
- Frederica Cassis, a psychologist who has worked on numerous WFH committees
- Woet Gianotten, a psychotherapist who is a senior lecturer at Utrecht and Rotterdam in the Netherlands where he lectures in medical sexology, presented this session.

Men and women living with a bleeding disorder may have a number of concerns that affect their relationships and intimacy.

For men there is the management of different bleeds during intercourse, even when they treat by prophylaxis, the management and treatment of HCV and HIV, and pain management. Self-image can also be a complex issue, due to people's own beliefs around having a bleeding disorder.

For women the issues can involve facing cultural prejudices around being a carrier of the bleeding disorder gene, marriage, family planning, pregnancy, postpartum bleeding, heavy periods and pain, post-coital bleeding, nose and gum bleeding, bruising, self-image, and perception.

When disclosing a bleeding disorder there can be feelings of fear and anxiety. Using Cognitive Behaviour Therapy can help with managing fear and anxiety. There are four components to this:

- **Cognitive** – negative thoughts, catastrophising, black and white thinking, what ifs...
- **Physical** - stomach upset, shaking etc.
- **Behavioural** – avoidance etc.
- **Emotional** - anxiety, fear, depression...

Analyse the fearful situation by first asking yourself what kind of thinking you use more, challenge catastrophic thoughts, follow your thoughts to logical outcomes, normalise and see that it is not that bad. Write and describe the event, date, and your ideas and feelings. Then evaluate what went well, and what didn't. Test and be curious. Remember, it's important to find ways of not judging yourself through this process.

All four components influence each other, so if we can change one, the others will improve.

People's reaction to your bleeding disorder will be dependent on how you tell them about it.

Imago therapy has been developed through the study of relationships, over thousands of clinical hours, with a variety of cultures, religions, and sexual orientations. It has been shown to be very useful for difficult or sensitive information as it has a component that focuses on empathy. Imago dialogue is initiated when one partner asks for an appointment to speak and the other partner agrees to participate.

The three steps in Imago Dialogue are mirroring, validating, and empathising.

Mirroring

Mirroring is using I language. One person sends a message to convey their thoughts, feelings, or experiences to the receiver e.g. I feel, I love, I need etc.

In response, the receiver echoes the sender's message word for word, or by paraphrasing.

Harville Hendrix has said "When you

mirror each other, you both get to experience what it is like to have someone pay close attention to you, understand exactly what you have to say, and honour your uniqueness."

Make sure you avoid shaming, blaming, or criticising your partner. Instead, just talk about your own viewpoint.

Validating

"I know it took a lot of courage to tell me that"

"I could understand you wanted to tell me because..."

A partner doesn't have to agree with what is said, instead the beauty is in being able to hold the two realities and truly understand how the other person is feeling.

Empathising

"I imagine that right now you might be feeling anxious about telling me; is that what you're feeling?"

The receiver then asks if those are accurate feelings, which gives the sender another opportunity to expand on their felt experience.

Communication within a relationship

Let's talk about sex, because we are all sexual beings. However, how do we integrate that with having a bleeding disorder?

Some people worry that sex education promotes sexual behaviour in young people. However, studies show that it doesn't lower the age of masturbation or of intercourse. In the Netherlands, comprehensive sexuality education is common, and young people first have intercourse later than in most Western countries. They also have very low rates of teenage pregnancy and abortion. The global happiness research shows that Dutch youth are the happiest young people.

Globally, research indicates that 50% of boys have started masturbating at 13.8yrs.

This is important, because boys when masturbating can have blood in their semen/sperm, it is not abnormal, it is not dangerous and it doesn't need medication. Masturbation with too much force can cause subcutaneous bleeding, that's also not dangerous, but it can be disturbing. This can be prevented by using a lubricant, investing in imagination or fantasy, because boys and men are easily visually aroused, or by slowing down the process to have less physicality.

For women, masturbation can also have implications. The psoas muscle is used during sexual intercourse, and sometimes in masturbation, and bleeding into a psoas muscle is a problem. There is pain, and bleeding isn't easy to stop or treat. Prevention techniques will help. Try to keep the muscles of your back a bit relaxed, and get excitement from other sources, i.e. fantasy, vibrator, visual stimulation, etc.

Young people generally start kissing and intense cuddling between 11 and 15. This is important because this behaviour can also include love bites, which aren't a good idea if you have a bleeding disorder. This would be a good time to talk to your girlfriend/boyfriend about your bleeding disorder.

During high excitement and intimacy oxytocin levels go up, the pain threshold goes down, and people can easily forget the potential damage of an uncommon position or the pain of a joint. Maximum excitement can put pressure on muscles and too much of a burden on the joints.

While young women with bleeding disorders will suffer fewer joint problems than boys, many girls will suffer from prolonged vaginal bleeding, more fatigue due to anaemia, less oral sex, and fewer possibilities for genital sexual sharing. It's important for young women to be sufficiently aroused, or to use proper lubrication to prevent bleeding. Anal

penetration is sometimes used due to prolonged monthly bleeding, and it is important to use proper lubrication.

For men and women with bleeding disorders, sex can also raise issues with musculoskeletal health. These may include pain, reduced range of motion, a fear of bleeding, weakness, arthritis, and poor balance.

Forced extension at end range will pinch already inflamed synovium, and be a new source of joint bleeding. Arthritic pain from repeated bleeds is exacerbated not only by angular motion, but also by compression, and the grinding of one joint surface on the other.

Most of the positions used during sex: involve muscles having to produce force either while significantly lengthened or shortened, and/or require that joints be positioned in the extremes of range and maintained there for extended periods.

All sex is exercise and the required physical attributes are individuality, flexibility, strength, and endurance. Safe sex in this context means matching the way you do it to your physical status.

Physical difficulties can make it challenging to maintain a healthy sex life while recovering from a bleed, injury, or surgery. Adapt your sex life to accommodate to a target joint or muscle, maintaining or regaining your sex life as arthropathy develops by trying different positions, or considering a sexual aid. An intimate rider, a body bouncer, wedges or platforms, and toys can also be used to enhance experiences.

Above all, be kind to your joints and muscles, make sure that surfaces are soft, and don't be ashamed to be a sexual being.

Sport and Exercise

MĀHIA NIGHTINGALE-PENE

This session had four presentations about sport and exercise for those with Haemophilia. The topics of the presentations were:

- Sports Therapy in Haemophilia
- Feeling Better: A Psychosocial perspective
- Sport: How to choose?
- A revolutionary experience

Sport Therapy in Haemophilia

Sports Therapy in Haemophilia was presented by Thomas Hilberg. Hilberg spoke about the importance of physical activity for people with Haemophilia, saying that, "Exercise is medicine". Hilberg provided evidence from the work he does in sports therapy to show that exercise is medicine.

Sport therapy is an exercise therapy – based on the motoric skills such as coordination, endurance, strength, and flexibility, and includes behaviour-orientated components. These components are prescribed and controlled by a physician. The therapy is planned and supervised by sports therapists, and performed by the patient alone or in a group.

Hilberg and his team conducted sport therapy on 64 people from Germany with moderate Haemophilia A or B between the ages of 16 – 75 years over 6 months. To be included in the test PwH had to be non-regular physical training people with organic inflammatory diseases. There were six different tests, one of which being a questionnaire. These tests were conducted twice to show pre and post therapy results.

The results showed significant improvement in strength for both upper and lower extremities. The overall post-therapy results in terms of physical performance, such as mobility, strength and coordination, endurance, and body perception improved big time over 6 months. The results showed reduction in joint pain and improved physical function. The evidence from this study shows that exercise really is medicine.

Feeling Better: A Psychosocial perspective

Sylvia von Mackensen spoke about the psychosocial perspective of sport and exercise on people with haemophilia, and particularly the impact on children with haemophilia. Her topic was about feeling better for the purpose of quality of life.

Physical activity not only benefits the physical health of PwH, but also the social and psychological health, and overall impacting on the quality of life. Von Mackensen provided evidence that shows the outcomes and the benefits for children of being included in sports. The evidence shows that it is highly beneficial for PwH to participate and be a part of sports.

Sport: How to choose?

Axel Seuser's korero was based on his own data and investigations, and other clinical and biomechanical data. When choosing a sport to participate in you must conduct your own personal preparations, and look at those safer sports with lower injury statistics.

Personal preparations include lessening own fitness deficits and meeting the demands of the sport, working with a coach, and seeking professional advice. When playing the sport stretching is important as well as warming up - there are a lot of studies that show warming up does decrease injury rate.

The safest sports are those that have low acceleration and low joint loading, sports that have low risk and rates of injury, sports that match own fitness with demands of sport,

and non-contact sports. The sports suggested by Seuser are swimming and surfing.

A revolutionary experience

Clive Smith spoke about his personal experience of participation in sport and competing at the highest level. The title of Smith's presentation was A Revolution Experience.

Smith spoke about his life from pre-birth so that the listeners could understand what treatments during his life were available, and understand the challenges he had/has.

Smith has Haemophilia A with target joints in his left elbow and knee. The worst years for bleeds were when he was aged 7 - 9, when he often used splints, crutches, and wheelchairs.

When he was 9 or 10 the revolution began because prophylaxis had just become available, he had less bleeds, he could go away on trips, and he felt like a normal kid. Smith said that prophylaxis was not just life changing for him, it was more - it was a revolution.

Between the ages of 10 - 16, he was slowly able to play sports like football, basketball, and of course swimming. When he was a young adult between the ages of 16-24 he became less active and had injuries.

Smith tried a 10km run when he was aged 24 - 30, which caused him a lot of pain, meant he couldn't walk for 3 weeks, and resulted in him not running. As an alternative, he began to go to the gym, as instructed by his physiotherapist, to build muscle, which helped his ankle to function better. He began to grow a lot of strength by going to the gym and training, and became able to participate in competitions like sprint ironman. With the support of his family, physiotherapist, and the prophylaxis, he was able to participate in full Ironman competitions and receive medals.

This was an outstanding story about success by a person with haemophilia participating in a multi-event sport that has high demand on stamina strength and physical strength.

Overall, the four presentations highlighted the absolute positive benefits of sport and exercise for people with Haemophilia. Sport is one of those controversial topics for those with Haemophilia, and we are aware of those worries. With moderation, planning, and great consideration, sport and exercise can impact positively on those of all ages with haemophilia, even for those with inflammations. Who knows, you might be the next best Ironman!

Voices of Women – New Challenges Different Visions

BY THERESA STEVENS

Claire McLintock chaired this brilliant session and referred to herself as the Scotland - New Zealand version of Oprah.

Karen Fine, her father and brother both affected by haemophilia, and she a mother of two girls with factor VII deficiency, spoke of the tests that were required to secure a formal diagnosis. There were concerns regarding "the trouble getting to adulthood". She stated that there was a "very apparent bleeding issue that needed to be addressed". Karen suffered with stress, anaemia, tiredness, and no social life!

Both Karen and her daughter were tested at the same time, and at age 40 Karen started to receive treatment. This treatment lead to an increase in weight due to the medications and injections over a 5-year period. Due to her heavy menstrual bleeding, Karen decided to trial an Intra Uterine Device (IUD). However, during the insertion her uterus was pierced, which resulted in a hysterectomy, as this was the protocol to keep Karen safe.

Karen continued with her story of haemorrhage, further surgery, contracting hepatitis B, and Post Traumatic Stress Disorder (PTSD). She said that it took her two years to "get back in order". Her new focus is her family, her career, and her self-esteem, because of the effect on her family, personal relationships, and other professionals. Karen went on to say that, while as a five-year-old she usually felt "left out and rejected", when she was older and working she always felt drained, tired, and had zero focus. She had constant performance issues, so she would panic, and become even more tired and overwhelmed, which became isolating and saw her going to her room to cope, "to be a better her".

Joanna is a 41-year-old woman with factor 1 deficiency. She was diagnosed at birth when her umbilical cord did not heal. Her older brother was also affected. Joanna contracted hepatitis B after an infusion of cryoprecipitate, and suffered from anaemia once she started menstruation. At this time she commenced the contraceptive pill to assist with reducing her bleeding.

At age 13 Joanna suffered her first intracranial bleed, a subdural haematoma in the occipital lobe. At this point, a port was inserted for prophylaxis, which resulted in Joanna having a Pulmonary Emboli (PD). At age 35 Joanna was diagnosed with breast cancer, which she survived. She spoke about both the positive and negative impacts her bleeding disorder has had on her life, especially since there was "no normal kid activity". She said the positive effect was how the bleeding disorder brought her family closer together, while the negative impact was on her sister who was not affected by a bleeding disorder. Joanna stated it was very hard to meet people romantically and tell them about her bleeding disorder.

Culturally Responsive Practices (CRP): Interacting with Indigenous communities through Piritoto

Te Whainoa Te Wiata
The Haemophilia Foundation of New Zealand (HFNZ)
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Introduction:

In 2009 the Haemophilia Foundation of New Zealand (HFNZ) established a group for Māori people with haemophilia (MPWH), which at the time was known as the Rōpū a Māori word literally meaning group. Four years later in 2013 the name Rōpū changed to Piritoto which in its entirety translates to English as 'connecting and binding bloodlines'. The name which was given by a kaumātua (elder) carries with it the understanding that it is who we are, and it is what we are doing. Thus, providing our Māori group with not only a name, but a statement embedded with action.

Piritoto as a workshop produces a culturally responsive lens towards working alongside Indigenous people with haemophilia (IPWH), especially when it comes to something as significant as blood. In the Māori view, blood carries ones past, present and future, this highlights the importance of creating awareness around blood for those in outreach and care for MPWH. Hence, the workshop carries with it a goal of establishing a vehicle for in-reach support to assist with outreach. In addition, it presents a holistic approach to complement the social and scientific means already in use. A practitioner's basic understanding of a patient's identity and culture can enhance communication and better ensure an intended outcome. Therefore, imparting Indigenous knowledge to outreach workers and other practitioners will begin a journey for better interaction with the Indigenous haemophilia constituent and vice versa.

Objectives:

- To provide those working alongside MPWH with the tools to better interact IPWH
- To improve communication between patient and health providers
- To educate on Māori issues
- To develop a model that can be used outside Aoteroa/New Zealand

Image: Piritoto noho marae (camp) at the Kookiri centre, Whaingaroa/Raglan.

Conclusion:

The result of a blended society is the production of a dominant culture leaving the other subordinate, this in effect also subordinates the challenges of those within the non-dominant culture. Yet, since the workshops have started the feedback from those within Piritoto has been one of positivity. Barriers are still very much present, but appreciation is clear. However, it is acknowledged that workshops such as Piritoto are very confronting, thus creating resistance and a lack of acceptance. Nevertheless, the stories and truths must not be known, but understood in order for the struggles to fully be appreciated. This means being aware of those things which underpin the current state of a culture, for example:

- History
- Historic changes within a country
- Cultural hardships within a blended society
- Current challenges due to implications of the past.

Ultimately, there is possibly no perfect way to run this workshop as personalities and ideologies are always at play but it is about beginning the dialogue to improve practice. It has been noted that as much as the outreach workers are often working in-between the different factions, they are rarely in a position to influence change. Therefore, aside from improving delivery another future step is to address those within the medical practice of treating haemophilia. To close, the workshops are held with the positive intention of acknowledging that haemophilia does not choose cultures, but the culture of an individual should be recognized.

Next Steps:

- To improve delivery.
- Broaden the scope outside of outreach workers and take Piritoto to the medical practitioners and funders to engage with.
- To provide the Piritoto to those outside of Aotearoa New Zealand.

From left to right: Te whare tapawhaa (The house of four walls) (Durie, 1994), Piritoto at Te roro o te rangi marae, Rotorua.
Note: All participants have granted permission to the author for the use of these images.

Method:

The initial plan was to hold a four weekend one night and one-day course throughout the year breaking down in-depth, some of the basic concepts of what it is to be Māori while living with haemophilia. However, it was decided to hold two a year during HFNZ staff meetings. These sessions were conducted in a Māori way with the assistance of Mason Durie's Māori health model 'Te Whare Tapawhaa' (shown above). It was decided that the use of te whare tapawhaa would be beneficial as many of our outreach workers were already using it as a part of their practice. As such the basic idea of the model was already understood, it was/is then the facilitators role to delve deeper into the concepts via linguistic and cultural understanding.

Concepts covered were:

- Te orokohanga o te ao - The creation story
- Whakapapa - Genealogy
- Te hekenga mai ki Aoteroa - The main migration to Aotearoa / New Zealand
- Tikanga / Kawa - Protocols
- Tikanga / Kawa a iwi -Tribal differences in protocols
- Physical, Spiritual and emotional hardships of MPWH and various reasons why
- Issues around blood

As this type of workshop is more qualitative a Plus, Minus, Interesting (PMI) form was constructed to canvas participants about the workshop. In addition, two extra questions were added to the form, 'what are you going to start doing from now?' and 'what are you going to stop from now?'. This produced great feedback and even better insight. Some of the responses of participants can be seen below.

PLUS	It was personally useful to know where and how come I had lost my voice around my maori identity, this will be helpful in my clinical practice, pacing with a client's pace. Bloody awesome, and very inspiring.
MINUS	The session was way too long and sections covered too drawn out and narrow. The amount of prior knowledge required at times. It was difficult for us Kiwis to figure out some stuff (to our discredit perhaps…).
INTERESTING	I feel a bit of disconnection not knowing my own whakapapa to have lost even more must have its challenges. Whakapapa - I thought I knew what this meant! Already used my new understanding of this in my work! I feel a bit of disconnection not knowing my own whakapapa to have lost even more must have its challenges.
START	Take a packet of biscuits when visiting families. I also have it on my to do list to undertake a Te Reo course of some type.
STOP	Don't assume a client will want to be contacted by an HFNZ Outreach Worker. I am going to continually check that my Pākehā ideas are identified in my head and endeavour to seek a different perspective that works with Māori.

References:
Durie, M. (1994). Whaiaora-Māori health development. Auckland, NZ: Oxford University Press.

Piritoto

Haemophilia Foundation of New Zealand Inc.
HFNZ

Access to powerpoints used in both workshops are available via QR code or the link below:
https://bit.ly/2JRrseO

The third speaker was Rachiomane, a psychologist from India, where she facilitates workshops and chairs the psychosocial committee. She believes women are being heard through their support systems. An issue is arranged marriages; when and where and, of course, how much is disclosed.

There is a struggle to educate, as it is a very emotional topic. One issue is when to test carriers and bleeders. The carrier daughter has a shock for the future, she is different, and there is a sense of loss because she is never going to be 'normal'. From this stems anger, anxiety, fear, and confusion. There is a lot absenteeism due to heavy menstruation.

Audience participation

One question raised was how long to wait to get treatment, appropriately answered by saying that there is "a lack of articulation to get treatment". A change of attitude is required. Remove the pent up anger and get the education required to get the tools!

One speaker elaborated by telling the audience that there was not a lot of information back in 1977. A move to Boston in 1982 resulted in advocacy from General Practitioner (GP), nursing staff, and Social Workers (SW), who are all good help. Her advice to Health Care Professionals (HCP) is that doctors need to talk to patients in a listening way. Remember the patient often knows more than the doctors do. It can be a struggle for HCPs just to listen to patients, however, it is a partnership, a team effort to ensure the patient is returned to optimum wellness. At all times listen and look the patient in the eye as you talk to them. Own your own wellness and treatment, as "a lot cannot be known". Advocate for your own care, as this is crucial to get a receptive doctor.

Another speaker spoke of the cost of carrier testing, and the fact that after an arranged marriage people expect the woman to be pregnant within six months of the nuptials. There is no right to find out the sex of a baby, or whether to keep or adopt the child. In contrast, other countries encourage and understand the need for advocacy. The power of keeping records is extremely important.

This family travelled 700 kilometres to have their 6-year-old tested, as they believed they had a right to know – the daughter was confirmed as a carrier. "Haemophilia never ends". The daughter understood she was a carrier like her mother and stated, "It is okay I am happy to be a carrier like you" this ultimately made the family happy.

In Canada for example, discussion around heavy periods resulted in a lack of education. Laboratory tests were utilised to confirm carrier status. Important information was learned at summer camps and talking to other girls. It was found that this is where carrier status was accepted.

"We are there to hear their voices"

In the United Kingdom (UK) a person with type III von Willibrand Disease (vWD), was diagnosed at six months of age. Subsequently, her mum kept a diary, and this was very helpful, because the woman had been adopted, so she had no records of bleeding disorders in her family. She stated that sometimes you just needed to spell out the obvious to get your point across.

Claire stated that the challenge was giving the correct amount of information. Communication is the key, not just to patients, but also to each other. Empower WOMEN to be important.

Look at the website https://letstalkperiod.ca/, which has a self-assessment tool, and discusses normal vs not normal bleeding.

The period tracker http://www.sisterhoodapp.com/ , assists you keep track of your period.

"Good to have the ability to challenge doctors, BUT not be known as that difficult patient"

What is your Everest?

BY NICKY HOLLINGS

Louis Marolow is an accomplished, determined young man who is studying to be a molecular biologist. Louis was diagnosed with Haemophilia at 18 months, and for the past 18 years has been on a bypass agent to manage his inhibitors.

As a teenager, Louis had bleeds in his elbows that impacted on his confidence and self-esteem. He struggled with anger, frustration, avoiding people, and multiple hospital visits impacted on his education. Then he had a teacher at high school who had a child with Haemophilia. He finally felt supported and understood, and that he wasn't the only one living with Haemophilia.

With the support of his family, friends, and teachers, he has been able to achieve his dreams of becoming a molecular biologist. He disclosed to his bosses how a bleed affects him physically with not being able to use his arms or needing to be off his feet. They have been very supportive, and have demonstrated love and care. Louis has not needed to have a day off work since he started.

Megan is a mother of two boys living with Servere Haemophilia, in Nigeria, when her oldest was diagnosed there was no treatment in Nigeria. Megan presented on the stages that families go through to reach their Mt Everest summit.

Finding quality treatment for her child. For Megan this was her hardest climb. Being an African woman with a partner that wasn't able to acknowledge that there is a problem. To be living in a country where there is no treatment, and living with the fear that there may never be any. The financial impact of having a child with haemophilia, having to take time off work, getting to and from hospital, purchasing pain relief. That desire to protect your child, to know when it's safe for your child to play. Knowing that it is your responsibility to care for your child, and to know the possibilities for treatment.

Perhaps the most important stage for Megan was to create awareness, to let others know that there is a need. Megan did this by emailing every person on the World Federation of Haemophilia site to tell them that she had no treatment for her son. She built a network that would support her in advocating for available treatment.

Her advice: No matter what you face, never give up, keep looking forward. In the face of any obstacle have an inspiration.

Asraf is a father of a son with haemophilia living in Mauritius, where there was no treatment for his son. The paediatrician said that his son would be dead at 11. Thankfully, his son isn't dead, and was able to have his first treatment at 18.

Asraf was a driving force behind the establishment of the Haemophilia Association of Mauritius (HAM). The idea for this happened when he had a conversation with an African doctor, while looking for treatment for his son. In 2007 there was a meeting between parents, doctors, and a blood bank, which became the advisory group for establishing HAM. The barriers encountered included:

- Inadequate treatment
- No haematologist
- Inappropriate diagnosis equipment
- Lack of knowledge from health care providers
- Stigma and curses
- Unknown to the public
- No prior medication for treatment
- No education on haemophilia
- Absence of budget from government
- Unknown number of haemophilia patients.

By 2012-2013 HAM had a strategic plan, patient screening, a national register, public awareness, fundraising, medic alert bracelets, established treatment protocol guidelines, advocacy of CFC, media liaison, their 1st NMO training, and a WFH general assembly meeting.

In 2013, they won twin of the year.

Now, HAM have four fulltime staff, they're submitting projects locally and internationally for funding, and are accessing overseas training for nurses, doctors, physio, and pathologists. HAM are patient and parent centred, an outreach programme has started, they have 94 patients diagnosed, a scientific registry, and haemophilia is known to the public. They have also developed a good relationship with the MOH, begun monthly reporting of CFC, IU per capita has increased, and they are setting up a youth committee.

HAM feels that they have reached their Everest because they are now able to obtain and sustain treatment and funding.

Aye Aye K is a Haematologist from Myanmar. Myanmar has a population of 52 million, and there are just two HTC. In 2007, they weren't able to diagnose different types and severities of haemophilia, treatment was with plasma, and there were 300 registered members. In 2014, they started twinning with the UK. Accreditation happened following a visit to the lab to meet with a haematologist. The initial objectives of the volunteer membership were to:

- Translate the WFH publication within 3 years
- Physio training to learn assessing
- Learn about the importance of exercise
- Teach parents
- Introduce prophylaxis as donations come from WFH
- Have parents happy to let kids move more.

Prophylaxis is now used, thanks to WFH donations. Hydrotherapy began last year,

for city-based children due to distance, with training for practitioners in Thailand and UK, but there is currently no pool available.

There has been increased support from within the community, and a campaign to raise awareness from the government in the media 2017

World haemophilia has supported sisters of haemophilia patients screened for being carriers. Mothers and some clients have been taught IV access. The media have been invited to the centre so they can see the needs of the members. Currently, doctors are supporting psychosocial needs not just medical needs, so they are working towards an outreach programme that will include social workers.

When will technology revolutionise care

BY KARL ARCHIBALD

The 2018 WFH Congress in Glasgow, Scotland, was, in my opinion, one of the best yet, and I think that most people would agree. It had a certain optimism, enthusiasm, and excitement about it.

Most of this came from a greater focus on other bleeding disorders, advancements in therapies, and greater access to them.

This session identified and discussed ways the presenters, and their NMOs, use technology to provide better services to people with bleeding disorders.

Reeshen Pillay, a 25 year old with severe FVIII deficiency, from the South African Hemophilia Society, outlined what he felt were the key drivers to help provide better access to therapies, and to empower PWBD.

1. Knowledge
2. Information exchanges
3. Education
4. Training

He outlined how, through using social media and the educational tools around him, by using the WFH.org eLearning platform, and by taking a methodical and quantitative approach to using the data at his disposal, he was able to identify and create a better understanding of his homeland, which included:

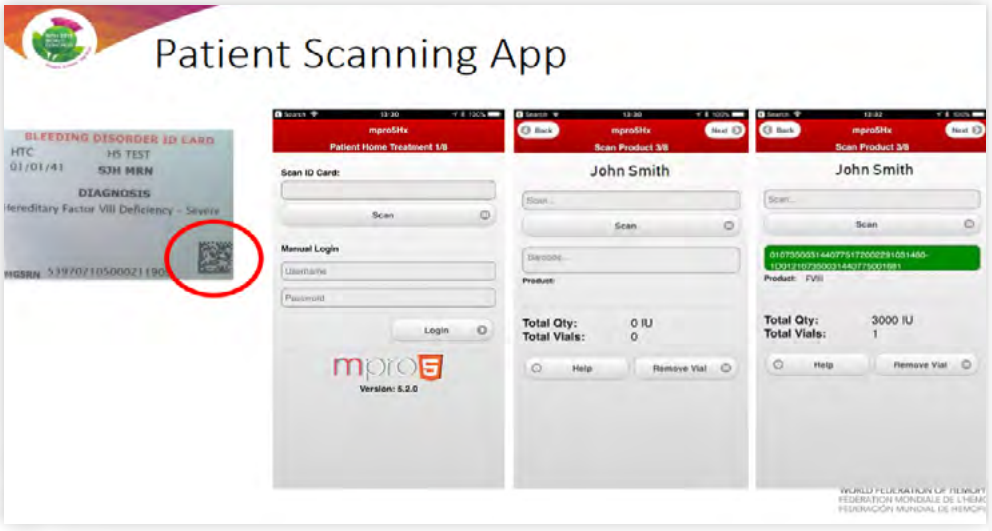
- A registry for clotting factor concentrates lowest diluent volume.
- An eLearning Module: Complications of Hemophilia and low risk of inhibitors.
- Using data from the 2016 WFH Annual Global Survey to benchmark progress.
- Utilising webcasts to managing patient expectations of new extended half-life products, and to identify the cost benefit ratios of these therapies.
- Attending a webinar: Options for Hemophilia in the Developing World.
- Taking this data, he started to identify what solutions were available to other developing countries, in order to achieve a better quality of care in his home country.

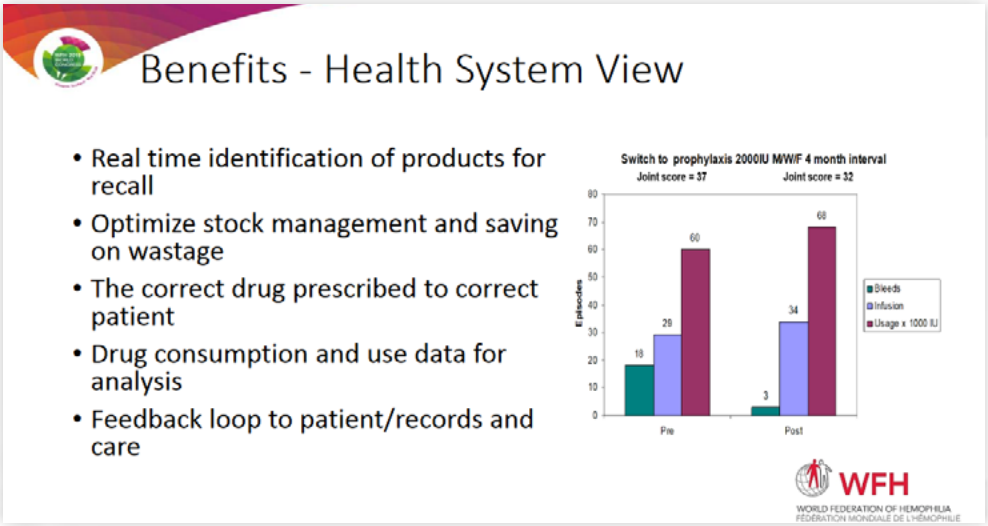
Reeshen concluded that through our digitally connected world we have access to the enormous WFH educational library (with more than 500 resources). This provides users with resources they might not have thought to search for, solutions to challenges that they might not have formulated, and ideas for new directions in which to take a project. More information on WFH resources can be found at **WFH.org**

Declan Noone was next up, talking about precision, personalised, patient care: The Irish experience.

Declan unfortunately was unable to attend this session - claimed he had a bleed, so he subbed in a friend to deliver the presentation for him. This was much at Declan's expense, and was delivered with the appropriate amount of Irish humour.

IHS undertook a study to identify and plan for the following two years of treatment by gathering as much data as possible. The





key questions to identify were:

- How much treatment are we actually using?
- How much do we need?
- Where are the "leaks"?
- How much benefit are we getting?
- How do we plan for the next 2 years of treatment?

They knew that one plan did not fit all, so by utilising data available to them through a mandatory factor tracking app, in addition to existing patient information, they undertook a '360 degree' review of the factory to factor process, and improved data collection at the same time. From a health system perspective, this was able to provide:

- Real time identification of products for recall
- Optimize stock management and saving on wastage
- The correct drug prescribed to correct patient
- Drug consumption and use data for analysis
- Feedback loop to patient/records and care.

However, from a patient perspective it provided confidence that the drugs received were:

- The prescribed drug at the correct dosage
- Genuine and safe product
- In date and fully active product
- Delivered to home monthly in good condition
- An adequate supply
- Easy to use

In addition, the app supported:

- Input to the clinical management of patients
- Instantaneous feedback to patient records on usage
- Dangerous bleeds highlighted to medical team
- Identification of recurring bleeds/target joints
- Identifying unusual frequency or over-treatment
- Possible early identification of inhibitors/incorrect dosage/target joint/new protocol required
- Input to regular check ups
- A tool to identify medical issues and appropriate further treatment

The overall vision of the Irish Personalized Approach to the Treatment of Haemophilia Study (iPATH) is *"to make fundamental discoveries in relation to haemophilia biology, thereby enabling novel approaches to patient stratification and ultimately personalized management of haemophilia and haemophiliac arthropathy."*

Shelly Reed followed, speaking about realising goals through effective social media campaigns.

Shelly comes from a varied background in digital media and social media data utilisation. She discussed how to run a successful marketing campaign and highlighted the impacts of social media, and the connotations that are associated with it.

The negative aspects of social media include:

- Less face-to-face communication
- Oversharing of information
- Requires constant vigilance
- Permanence
- A loose collective grasp of the truth
- Skewed perception

Not enough research exists currently to know all of the long-term effects. However, Shelley also pointed out the good. She states that it:

- Is a great marketing tool
- Is cost effective
- Is very accessible
- Shares media quickly and easily

- Is wide reaching
- Encourages the use of new technology
- Encourages more frequent communication
- Allows orgs to capture engagement (likes, shares, sentiment, demographic info, etc.)

So what is a Social Media Campaign? It is *"A coordinated marketing effort to reinforce or assist with a business goal using one or more social media platforms"*. Shelly used an example of what an NMO social media campaign could be used for:

- Events
- Education
- Advocacy
- Fundraising
- Needs assessment
- Build marketing list
- Awareness
- Improve brand engagement and perception.

Taking the needs assessment route, questions to consider could include:

- Who: audience, involvement
- When: beginning and end, timing
- Where: social media platforms
- What: objective, resources, metrics
- How: plan, execution, evaluate

Best Practices for a Successful Campaign:

1. Conduct a needs assessment
2. Set an attainable goal
3. Develop and implement a plan: strategy, timeline, resources, challenges
4. Promote across multiple channels
5. Monitor performance and adjust strategy, if needed
6. Analyse results and evaluate campaign

A post-analysis of the campaign will allow you to identify and monitor engagements, reactions, shares, and comments. Good analysis allows you to learn which posts get the most engagement and why, and provide an opportunity to take lessons learned to apply to the next campaign.

Milena Pirnat then spoke about the benefits of online patient communities.

Milena is a #Hemomom who created an online community through Facebook specifically designed to:

- Understand and identify with the supports for Haemophilia
- Rethink networked caregiver care
- Explore the potential and benefits of an online social media community.

Her Facebook group, Hemophilia Mother, is an open group for women with bleeding

disorders, mothers, and all women that are related to children, brothers, and fathers who have haemophilia and other more rare bleeding disorders.

She started the group in 2011 because she felt isolated in her new home, Canada, after fleeing a war zone and gaining refugee status. She was a motherless mother, shocked from diagnosis, with an emotional longing to connect to others with similar experiences. She states that she had guilt of transferring the gene, the anger of not knowing, and, as a physician and health educator, it made sense to her to open a group to offer support and education.

Her page has since grown to over 6800 members globally who find the kind of support offered there useful in their everyday lives and health management.

Milena said that the benefits of this group being open are:

- It's easier to be found by new mums
- It raises awareness about bleeding disorders
- Enables education of the wider population
- Provides humour and an outlet
- Provides a support system who are indirectly aware of how you feel and what you are dealing with
- Helps with the understanding of the global situation.

However, Milena also points out that, as with all social media, there are some disadvantages to an open group in a public forum.

Cons:

- Concerns about privacy
- Being uncomfortable sharing openly, being vulnerable
- Soliciting, selling, and promoting
- Unintentional but well-meaning medical advice
- Other groups using information on the site for their own purposes.

Ultimately, this group has provided real benefit to its members. Milena left us with a quote from one of the unnamed members, which she stated captured the essence of her group and why she started it. I feel it is equally fitting to finish with:

"Make no mistake this is not a Facebook group. This is A PERFECTLY FUNCTIONING SUPPORT GROUP that has spread its wings all over the world. A support group that normally I would not attend in real life but interestingly the internet reality that I never believe in actually showed its power here, and, instead of getting up and going personally to a support group, this online availability was the reason I could be reached by help".

Youth Twinning: New Zealand

PRESENTED BY ASHLEY TAYLOR-FOWLIE AND DEON YORK

Written by Ashley Taylor-Fowlie and Lauren Nyhan

The Application Process

The application process was really a stab in the dark for us. As we went through and listed why we thought we should be selected for Twinning it was actually an amazing self-realisation process.

We realised that we had great members who were talented and committed and who were willing to share those talents within our own regions and country with the rest of the world.

It made us proud to put forward such an application and to appreciate fully the range of skills that our members brought to the table.

Twinning with Nepal

Chairperson, Lauren was excited to hear and share the news that we had been chosen to twin with Nepal. Our excitement was just through the roof, as we could not believe that little old New Zealand had been picked. We have this thing in New Zealand where people are very humble and quite shy about achievements, so we tend not to put ourselves out there. However, this time it was totally worth it!

Then, our excitement turned to nerves. How were we supposed to help organise a twinning project with a country that was at least three flights away! The responsibility we felt was also huge. Not only to our committee and ourselves, but also to HFNZ, to Nepal, and to WFH.

Assessment Visit

Day 1: Assessment Visit

Day 2: HTC Visit, NHS Head Office, and Government

Day 3: Planning

Concerns during the Visit

Courtney and Hemi reported that the language barrier was scary before they got to Nepal, but apart from a few nuances with Kiwi vs Nepali English things seemed to go pretty smoothly, having an open mind, patience and listening to understand rather than to respond were key to overcoming this obstacle.

It was difficult for our assessors, coming from a country with such progressive treatment, to see the hardships faced by the Nepali people. However, they had to be very clear in their mind, and we have reflected on this since they got back, that the purpose of the twinning programme was not as advocates for the Nepal Youth Committee, but rather to share the skills and strengths that each of the groups had, and to learn from one another. That was actually very hard, as Kiwis with a 'can do' attitude who just want to get stuck in and make things equal for everyone.

Twinning Goals

We collaborated with other members of the HFNZ Youth Committee about what our vision was for this twinning project. It was easy for us to get carried away about all the things we thought we should do and achieve. After an excited planning session, we took a step back and realised that we needed to put ourselves in the shoes of the Nepal Youth Committee - this was after all a twinning project! We sent our ideas off to Nepal and waited to hear back, hoping we'd got it right! Luckily, Hemi and Courtney had already done some ground work about

what the committee in Nepal wanted, so we were all on the same page.

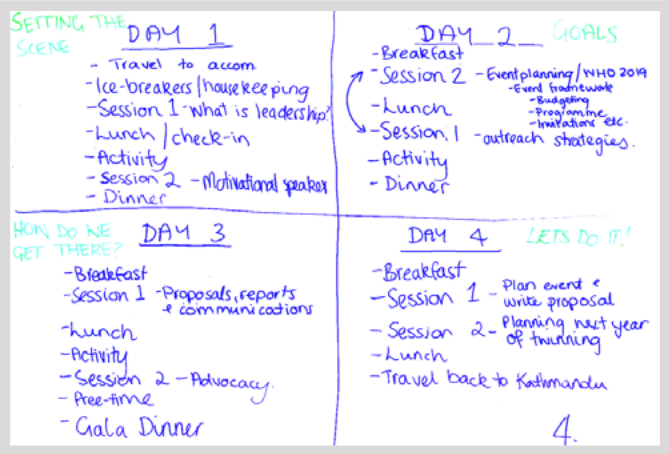
What has been achieved

So far, we have held two planning workshops and kept in contact with Nepal about activities, ideas, programmes, and venues etc. This is the most fired up the committee has ever been about a project, and these have been some of our most productive meetings.

We have planned to head to Nepal at the end of September/beginning of October to facilitate a youth leadership training retreat.

Leadership Retreat

In collaboration with Nepal, we intend to offer a Leadership retreat set for September/October 2018. This is what our plan for the retreat looks like:



Women and bleeding disorders: Clinical and Psychological issues

BY LYNNE CAMPBELL

Speaker: Claire McLintock - Haematologist and Obstetric Physician at National Women's Health, Auckland City Hospital

In recent years, the impact of bleeding disorders on women has been recognised. However, with so many life-changing advances in the treatment of haemophilia, it is easy to overlook the clinical and psychosocial impacts of bleeding in women. It is imperative that medical teams are mindful of, and attentive to, specific women's issues. Gradually a better understanding of the physical, social, and emotional challenges that women with bleeding disorders face in their day-to-day lives is being validated.

McLintock provided a brief summary of the inheritance patterns for haemophilia, and an illustration of the lyonisation of the X chromosome in women, where, because females have two XX chromosomes and randomly only one X of the two is activated or silent, a skewed x-inactivation can cause bleeding in women.

Claire noted that historically in the inheritance of haemophilia, a sex-linked mutation on the X chromosome, "it may almost be said that the females hand down the disease and the males are the victims of it".

Although there have been tremendous advances in addressing the impact of bleeding on men in recent years, the needs of women with bleeding issues have been largely ignored.

It used to be thought that only males get haemophilia, and that women are simply "carriers" of haemophilia. However, for every male with haemophilia, up to five females in each family need to be tested to find out if they have the gene.

In reality both males AND females carry the gene for haemophilia from one generation to the next and clotting factor levels vary.

The risk of excessive bleeding in women resulting from inherited bleeding disorders such as vWD and haemophilia are now proven, and this has given rise to attention being afforded to the physiological and psychological impacts of excessive bleeding in women.

For many women with vWD, and some women with the haemophilia gene, abnormal uterine bleeding (previously known as menorrhagia) can now be described as acute or chronic depending on quantity, duration, and timing of bleeding.

It is imperative women are given the opportunity to evaluate the extent of their bleeding by questioning the type and quantity of sanitary products they need to use, concerns about leakage, time off work or school, and whether anaemia has meant they have needed iron supplements and/or blood transfusions.

Essentially excessive menstrual blood loss impacts negatively on the physical, social, emotional, and material quality of life for some women.

After considering a range of negative cultural practices, taboos and mores surrounding menstruation in other cultures from around the world, by contrast McLintock focussed on the very positive, spiritual, and sacred Maori descriptions for menstruation in Maori culture. The words used were revered, and described menstruation as a time to rest; for example

tapu (set apart/sacred), tohi whakatapu (restriction- where women were to rest from gathering food), and whare tangata (a time for meditation at sacred sites). It was noted that in some Northern American tribes menstruation was also considered to be sacred.

Fundamentally, in order for women not to be isolated in today's world, the topic of menstruation needs to be discussed openly.

McLintock referenced the books: Blood Magic -The Anthropology of Menstruation plus Te Awa Atua -Menstruation in the pre-Colonial Maori World, written by Ngahuia Murphy.

She also drew the audience attention to The HOW Collaborative (a collaboration between Australia and New Zealand) for Haematology in Obstetrics and Women's health which aims to achieve excellence in health outcomes in women with blood conditions related to pregnancy, birth, gynaecological and reproductive health in Australia and New Zealand.

The time has come for the needs of all women with inherited bleeding disorders to be addressed.

Youth Workshop

BY ASHLEY TAYLOR-FOWLIE

To start the Youth workshop, we highlighted previous WFH Youth Workshops held at Congress. In 2014 we looked at Youth Engagement in Action: Sharing of Best Practices and Experiences, 2016 was Transitions: Sharing Personal and Organisations Experiences on Key Youth Issues; and now, in 2018, the focus is Youth Collaboration in Action: Sharing of Personal and Organisational Experiences

The Objectives of the 2018 Youth Collaboration in Action Workshop were to:

- **Share** personal and patient organisation experiences on the theme of 'Youth Collaboration'
- **Learn** about the WFH Youth Programmes and Activities
- Share **Tips** on how the youth can navigate through Congress and maximise their time
- **Brainstorm** other youth initiatives and activities that could be developed by the WFH to meet the needs of youth with bleeding disorders.
- **Meet** other youth and the chance to network and learn from each other.

WFH Youth Programmes and Activities:

The aim of the programmes and activities provided by the WFH are to empower the next generation of leaders in the bleeding disorder community and to ensure that the youth are well prepared to take over a leadership role.

- **WFH Youth Fellowship Programme:** Is a bi-annual programme since 2004. Consisting of an international competition and allowing the recipients to participate in the WFH Global NMO Training, World Congress, and Annual Meeting of the General Assembly. To date, 147 Youth Fellowships have been awarded, and 24 of these fellows were in Glasgow. To apply for a Youth Fellowship (take note for those who are interested in applying for next Congress) applicants must be a person with a bleeding disorder aged between 18 and 30 years. Application forms and documents supporting the applicants are sent to the NMOs. The NMOs then select the top candidate for country nomination. Candidates are then scored and reviewed by the WFH committees. Top scoring candidates are awarded fellowships, with three alternates also being selected.
- **The Susan Skinner Memorial Fund Scholarship:** Was established in 2007 by WFH USA to support the training, education, and leadership development skills of young women with bleeding disorders. The recipient must be of age between 18-30 years. There needs to have been demonstration of outstanding leadership to improve the care of women with bleeding disorders in their country and the potential to become future leaders in their bleeding disorder community. Candidates are selected from America and other countries around the world.
- **Advocacy:** The WFH holds interregional workshops to help NMO leaders improve advocacy efforts.
- **Global NMO Training:** A 3-day workshop held every 2 years before Congress for NMOs, also involves the youth meetings.
- **WFH World Congress:** Youth sessions have been involved at Congress since 2014.
- **Youth Leadership Development:** 2-day highly interactive workshop with minimal lectures and maximum time devoted to discussions and small group work.

First Workshop - 2013 Montreal Canada:
12 participants from 11 countries and 29 individuals through online broadcasting. Topics included; why should advocacy matter to youth, building strong leaders, engaging youth in your NMO, influencing public policy, creating advocacy campaigns using social media and sharing of best practices session, which included youth group challenges and achievements.

Second Workshop - 2016 Barcelona, Spain:
22 participants from 21 countries and 100+ engaged through the Facebook live event. Topics included; youth engagement,

challenges, cycles of youth engagement, presentations from youth groups, WFH resources for youth, future youth programmes such as youth twinning and training on social media.

Third Workshop - 2017 Panama City, Panama:
17 participants from 15 countries and the Facebook live event had 925 views. Topics included; youth engagement, advocacy, project planning, social media WFH resources for you and first-time coverage of sensitive topic such as transitioning into adulthood, disclosure of a bleeding disorder, communication skills, relationships and sexuality.

WFH Youth Committee

This committee was established in 2014. The aims of the committee are to guide the WFH on youth leadership issues, develop activities for the NMO training programme, and develop education materials generated towards young youth leaders.

The members of the Youth Leadership Operating Committee for 2016-2018 are:

- Jeshua van Dejick (Netherlands)
- Nikole Scappe (USA)
- Alex Parkinson (Jamaica)
- Luis Manuel Melgar (Panama)
- Raissi Aziz (Tunisia)
- Firaoli Kumbi (Ethiopia)
- Harshal Kale (India)
- Chanthearithy Run (Cambodia)
- Ashley Taylor-Fowlie (New Zealand)
- Deon York, Chair (New Zealand)

The mandate of the WFH Youth Committee is to:

- Provide Advice: Through identifying areas where the WFH can incorporate Youth Leadership development strategies through programmes and activities
- Serve: Through advisors, authors and reviewers of educational materials
- Promote: Interest and involvement in WFH Youth Leadership activities
- Participate: Through involvement in GNMOT working group conference calls and evaluate applications for youth fellowships, and by participating in youth related meetings (congress and international/ regional/national youth events)

WFH Twinning Programme

WFH's Twinning Programme was established to help grow bleeding disorder services in countries where support and advocacy was less developed, or non-existent. Organisations who were more established collaborated with those that

were less established to share knowledge and information. The general timeline of the Twinning Programme is:

- 1994: Start of HTC Twinning. First regional HTC Twinning
- 1995: Start of HOT Twinning Programme
- 1999: First Twinning partner graduated from 'emerging' to 'established'
- 2018: Launch of the new component of the WFH Twinning Programme, Youth Group Twinning

The newest twinning initiative is Youth Group Twinning. Here, established countries work with youth groups connected to emerging bleeding disorder organisations in less established countries. The first twins are Canada with Bangladesh, USA with India, and New Zealand with Nepal. The New Zealand programme is unique in that the National Youth Committee, rather than the national organisation, are managing the twinning.

While the programme put together by each group is specific to the needs of the emerging organisation, there are some broad areas of focus. These areas include:

- Leadership Training
- Summer camps
- Good governance activities of youth groups
- Training workshops (peer support groups, social media, public speaking)
- Support advocacy initiatives
- World Haemophilia Day activities

Young Voices

Young Voices is a series of articles, available online, designed to help identify, mobilise, and empower future leaders to participate in the global conversation and effect positive change in their communities. Each of the 14 articles provides positive and practical information to a specific area of youth involvement. You can read and download these articles at www.WFH.org/YoungVoices.

Youth Presentation Panel Discussion

WFH Youth Twinning Pilot Haemophilia Federation India and National Haemophilia Foundation USA by Marlee Whetten

- NHF have a National Youth Leadership institute, which is a 3 year programme consisting of 27 members, both male and female, including patients and siblings.
- India Youth's Programme Objectives are; hold 4 regional camps and annual youth group training, identify the second line of future leaders, build on leadership skills for future leaders, youth to be educated, informed, and involved.
- The Twinning Programme will address team building, presentation skills, working with your NMO, introduction to advocacy, and discovering your strengths.

Fundraising Activities of the Lebanese Association for Haemophilia by Rana Mahmound

- Fundraising activities included a special food market, which helped small restaurants to sell their local food and donated the profits to different associations.
- Gatherings every week in different cities with the possibility to raise money for different associations.

Youth Collaborations and the psychosocial activities of the Kenya Haemophilia Association by Carlos Odera

- The Youth Objectives are to create awareness of the public on haemophilia and other bleeding disorders through involvement in World Haemophilia Day celebrations, media engagement, and public fora.
- The Core Values are Inclusiveness, Connectedness, Advocacy, Respect, and Excellence.

Collaboration through Social Media by David Braun Brazilian Federation of Haemophilia

- Collaboration through social media to create advocacy, awareness, and education.
- Important for youth to take advantage of social media aspects to create connections and achieve common goals.
- Our message is to go beyond just giving patient information but inspire them to their life to its fullest
- Can follow David's story here:
    - Youtube: David Brownie Vlogs or Fusca Ungido
    - Instagram/Twitter/Facebook @ DavidBrownie
    - Brazilian Hemophilic Federation @FederacaoBrasileiraDeHemofilia
    - http://www.hemofiliabrasil.org.br/

Other stories shared included:

- My road to Emancipation through Advocacy and Engagement by Mathieu Jackson
- The importance of Resilience by Nikole Scappe
- Haemophilia does not define who I am by Jack Grehan

Regions and Groups

HFNZ's Regional and Group committees enable all our members to participate in the running of the Foundation, and to connect with and support one another. Each region and group 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.

Central

By Ashley Taylor-Fowlie

The Wellington fishing charter that had been planned for late 2017, and was postponed due to the weather, finally took place at the end of January 2018. Originally, this event was intended to be for men with a bleeding disorder. However, with low numbers, we extended this to any men and accepted some requests to bring children too.

This was a great day for those who did attend, even though the fish were not keen on being caught. The weather was fantastic and the conversation lively, and most of us were able to return home with fresh fish for dinner.

We had our first committee meeting of the year on Sunday 25 February to plan further events for the central region.

These include a quiz night for World Haemophilia Day. Planning is also underway for a youth event as well as a camp in Napier on 1-2 September. Our last camp was held in 2016 so we are due a winter escape together as a region. Keep an eye out for your invites in June. And if you have any suggestions for events you would like to see in our region please drop us an email at hfnzcentral@gmail.com

Midland

By Tineke Maoate

A warm and cosy hello from the Midland whānau. For all of you who were able to attend Joy's farewell, thank you for coming. It was an amazing night for us to thank her for all her hard work and support.

Thank you too, to all our members that attended our AGM at Rocktopia in Tauranga. The children had a wonderful time climbing all the different challenging structures and rock walls. The outcome of the AGM is that we have new office holders, and a new committee, who have already shared some cool ideas for future events. If you have any ideas please let us know.

Remember we are here to support each other. Hope to see some of you women at the Women's Weekend event in Wellington in September. All take care and I look forward to seeing everyone soon.

Northern Report

By Neil Smith

Hi all.

Things have been reasonably quiet in the Northern Region while we hunker down for winter.

Our latest outing was to a northern region special of Mongolian bbq. We had a really great attendance and appreciated just how diverse our membership is, particularly with a few smaller ones that have joined the whānau since we last met. For those that don't know, a Mongolian bbq restaurant is all you can eat, pick your raw food and they'll cook it on a huge hot plate in front of you. I think we certainly got our value for money and I doubt they've been through as much ice cream and chocolate chips thanks to our Mongolian horde of children.

This event also doubled as our informal AGM so a nod to our outgoing committee and in with the new. That's minus me I'm afraid while I focus on three (crikey) of said horde of children.

Like all HFNZ branches, Northern is managed by volunteers. Currently we only have a very small number of active volunteers, plus an awesome Outreach Worker, as part of our team. Following the AGM, only two people put their hand up, so there are still places available on our committee. If you're interested in joining us, at any point, or are able to help in any way please let Nicky (Northern Outreach Worker) know.

The Committee meet four or five times a year and organise events like our recent dinner. If we don't have a committee then we don't have events. You can be as involved as you like, depending on how much time you're willing/able to contribute. It's a great way to get closer to what HFNZ do, influence what events we hold and give a little back.

We really do need a hand in the committee for organising events. Otherwise, they are just not going to happen.

Event wise coming up I'm pleased to say we are off to Rainbows End in November so look out for details.

As my grandpa used to say TTFNQ. (Ta ta for now)...and the Q is for fish and chips.

Piritoto

By Tuatahi Pene

E rau rangatira mā, nei rā te mihi kau atu ki te hunga kai-panui

Hello, Bloodline readers!

One of the most talked about topics of 2018 in the Haemophilia community would have to be the World Haemophilia Congress in Glasgow. The global congregation consisted of a network of bleeders from over 140 plus countries plus and a range of multi-disciplinary professionals all coming together to provide the latest information of medical findings and research, as well as an occasion for people to get familiar with each other.

Piritoto was fortunate to be able to send six members plus a teeny tot along to the congress. Two of our members, Te Whainoa Te Wiata and Māhia Nightingale-Pene, stood to present abstracts on behalf of HFNZ to congress members, with the other members taking the opportunity to be part in the festivities and learning atmosphere. The abstracts were submitted as an informative discussion to allow members from all over the world to provide useful insights to what may be happening in their own countries, a new technique in procedures, or simply have another outlook on previously discussed topics.

Te Whainoa spoke about our MRG and how we are focused as a whānau to help those who identify themselves as Māori living with Haemophilia. His presentation showed the many activities the whānau are doing, from the Noho to Matariki celebrations and so on. The presentation was on how to engage with indigenous people of a land to best start a dialogue for cultural responsiveness.

Māhia presented on menstruation in carriers and women with bleeding disorders. The discussion focused on how to change the attitudes about how heavy menstrual bleeding in women with bleeding disorders is treated, looking at what is necessary to, first, recognize the problem, and then provide the necessary steps of treatment.

Altogether, everybody wove themselves in and out of the many activities taking place at the congress, from the fantastic welcome ceremony where men wearing kilts played the bagpipes to the masses to the workshops, seminars, and group discussions.

The worldwide information on haemophilia has moved from a small narrowed view to a wide range of multi-disciplinary approaches, the explosion of new techniques of treatment and new theories have broadened the horizon for Haemophiliacs. The learning that takes place at each congress has encouraged the coming together of people from all walks of life, and from many different disciplines. A big thank you must be made to all the organisers, the facilitators, and especially the people of Scotland, who opened their country to our community. They very much made us feel welcomed in their own land.

Mā te wā whānau.

Southern Report

By Karl Archibald

A wee bit has been happening in Southern over the last few months.

Ali, our outreach worker, has moved on. However, where one person departs a new opportunity is created. I am pleased to welcome Ross Paterson to the role of Southern Outreach Worker, Ross comes from a varied background of support and mentoring roles and I am sure he will succeed in our region. Ross started on July 30. I am sure you will make him feel welcome.

Southern hosted its annual fundraising play in Christchurch on May 5; this was great with just under 100 people attending. There were some amazing raffles, and the behind-the-scenes effort that made it happen was very impressive. A special mention to Bo Silver and xyz for all their hard work.

On Friday June 15, the Christchurch office officially closed in preparation to move away from the mainland to Wellington. A fantastic farewell to the office was had at Winnie bagoes Christchurch on July the 21st.

Saturday the 21st of July also played host to the Southern Regional AGM where new committee members have come on board to lead the Southern regional committee for the next year. I have stepped down from the role of Chair.

Thank you for your support over the last 12 months.

Youth Report

By Lauren Nyhan

The National Youth Committee has been hard at work over the past few months preparing and planning for the Youth Leadership programme we are facilitating in Kathmandu as part of our twinning

project with Nepal. The programme covers topics such as advocacy, effective communication, and planning. With two face-to-face meetings under our belt, the plan for our next meeting is to finalise plans on the ground in Kathmandu, including the potential inclusion of a translator to ensure that all participants get the most out of the sessions.

Ashley and Deon gave a successful presentation at World Congress in Scotland earlier this year regarding the twinning project. We are hugely looking forward to hearing back about the ideas shared by other twinning partners. We would like to thank and congratulate both Ashley and Deon for their successful presentation, and for representing HFNZ and the Youth Committee.

In the regions, Central youth are looking to host an informal gathering at Orlando Country club with a bite to eat and a few hits of the golf ball. Absolutely no talent is required so keep an eye out in your inbox for the dates and details.

Finally, we would like to say goodbye to the bricks and mortar that is the Christchurch office. There were many cups of tea and stories shared out of the premises, and we're sure there were more than a few sad faces when it was announced that we would be moving out. However, we are optimistic about the Foundation's plans for new premises and want to thank Richard and Phil for being as helpful and responsive as ever in their new offices at home.

News from around the world

Cardiovascular Disease Still a Risk in Moderate to Severe Hemophilia Patients

BY IQRA MUMAL

Although moderate to severe hemophilia patients are generally protected against the development of cardiovascular disease, cardiovascular-related events can still occur in this patient population, a study reports.

These findings suggest that efforts should be made to prevent cardiovascular disease in hemophilia patients who have risk factors associated with the condition.

The study, "A cross-sectional analysis of cardiovascular disease in the hemophilia population," was published in the journal Blood Advances.

Hemophilia A and B are bleeding disorders caused by an absence or decrease in the levels of the coagulation factor VIII (FVIII) or factor IX (FIX), respectively.

Out of the 14,990 men with hemophilia A or B in the American Thrombosis and Hemostasis Network data set, 14.5% are 50 years or older. Because the risk for cardiovascular disease increases with age, it is important to study this condition in older hemophilia patients.

Many cardiovascular events occur when a blood vessel is blocked — most often by a blood clot. Because of low levels of the FVIII and FIX clotting factors, hemophilia patients are thought to be more or less protected from these issues.

However, recent evidence suggests that hemophilia patients can suffer from atherosclerosis, or plaques in the arteries, at a similar rate as the general population. Furthermore, risk factors that contribute to cardiovascular disease, such as hypertension, or high blood pressure, are frequently present in men with severe hemophilia.

To date, most studies that have investigated the relationship between cardiovascular disease and hemophilia have evaluated patients at all levels of hemophilia severity. Very few studies have focused only on patients with moderate and severe disease, who would theoretically have the most protection.

Therefore, researchers conducted a study to determine the prevalence of cardiovascular disease and risk factors in older men with moderate and severe hemophilia in the United States. They evaluated 200 patients, between the ages of 54 and 73 years old, at 19 U.S. hemophilia treatment centers.

Similar to other studies, results showed that cardiovascular disease risk factors were common in men with moderate and severe hemophilia. In particular, more than 50% of the participants had dyslipidemia, an abnormal amount of fats in the blood, and hypertension. Additionally, nearly one-third of the patients were obese.

Interestingly, researchers found that only 36% of patients with hypertension in the study were receiving antihypertensive medication.

"It is important that hemophilia treaters inform patients and their primary care providers of the increased prevalence of

hypertension in hemophilia, screen for hypertension at hemophilia clinic visits, and facilitate appropriate care," the authors wrote.

Next, researchers investigated the overall rates of cardiovascular disease in this patient population. Results showed that 15% of hemophilia patients had cardiovascular disease, compared with 25.8% of men of similar age. Therefore, hemophilia patients have significantly less cardiovascular disease compared with age-matched controls, suggesting a protective effect.

However, the authors said, "on an individual patient level, CVD [cardiovascular disease] events occur and efforts to prevent cardiovascular events are warranted."

Researchers suggest that secondary preventive therapy should be considered for men who have risk factors for cardiovascular disease, including statins for patients with dyslipidemia and low-dose aspirin for patients with hypertension.

Source: https://hemophilianewstoday.com/2018/07/09/hemophilia-patients-still-at-risk-cardiovascular-disease-study/

First Patient Enrolled in Phase 3 Trial of uniQure's AMT-061 for Hemophilia B

BY CATARINA SILVA

UniQure has enrolled the first patient in its Phase 3 HOPE-B single-dose trial investigating the effectiveness and safety of AMT-061 in treating people with severe or moderately severe hemophilia B.

AMT-061 uses a viral vector to deliver the gene for a mutated clotting factor IX (FIX), which is also known as the Padua variant (FIX-Padua). This man-made mutation leads to a higher production of FIX, which is compromised in hemophilia B patients.

"AMT-061 has the potential to be a major advancement in gene therapy for patients affected by hemophiliaB," Steven Pipe, MD, pediatric medical director of the hemophilia and coagulation disorders program at the University of Michigan and principal investigator of the HOPE-B clinical trial, said in a press release.

"A one-time treatment, such as AMT-061, could be life-changing for these patients, many of whom struggle to manage ongoing challenges, including compliance with frequent infusions, and recurrent episodes of bleeding," he added.

The Phase 3 HOPE-B trial (NCT03569891) is a multi-center, open-label, single-dose study evaluating the safety and effectiveness of AMT-061.

Researchers hope to recruit a total of 56 severe or moderately severe adult hemophilia B patients for the trial. Initially, participants will be followed for six months while on their standard of care therapy to establish baseline control of factor IX activity levels.

After that, they will receive a single intravenous administration of AMT-061. Dosing is expected to begin early in 2019.

The trial's primary goal is the assessment of FIX activity levels following the single-dose administration of AMT-061.

Secondary endpoints will compare the annual bleeding rate between standard of care treatment and AMT-061 as well as annualized factor IX replacement therapy use rate. A five-year follow-up is also planned to assess any adverse events.

UniQure is also initiating a short, Phase 2b dose-confirmation trial of AMT-061 (NCT03489291) in just three adult patients, also with severe or moderately severe hemophilia B. Enrollment is expected to begin this month (July).

Participants will receive a single dose of AMT-061. They will be followed for six to eight weeks to determine FIX activity levels and to confirm the dose of AMT-061 for the Phase 3 HOPE-B study. The three dose-confirmation trial participants will continue to be observed for one year.

"We are delighted to have enrolled the first patient in this Phase III pivotal study of a gene therapy for patients with hemophilia B," said Steve Zelenkofske, DO, chief medical officer at uniQure. "This represents a significant milestone for uniQure as we advance a potentially best-in-class gene therapy for patients with this life-altering disorder."

"In addition to advancing our pivotal trial, we have also initiated patient recruitment for our Phase IIb dose-confirmation study and expect to commence enrollment in July. We look forward to announcing top-line FIX data from the dose-confirmation study before the end of the year," Zelenkofske added.

In 2017, the U.S. Food and Drug Administration granted breakthrough therapy designation to AMT-061 based on results from the Phase 1/2 study of its sister gene therapy, AMT-060.

Also last year, the European Medicines Agency (EMA) granted Priority Medicines (PRIME) designation to AMT-061, which helps accelerate the development of medicines that target an unmet medical need.

Source: https://hemophilianewstoday.com/2018/07/02/first-hemophilia-b-patient-enrolled-phase-3-trial-amt-061/

Hemophilia Drug Development Summit Takes Aim at Hurdles Facing New and Upcoming Treatments

BY ALICE MELÃO

Key leaders in hemophilia therapy development, representing large pharmaceutical and biotech firms as well as academia and contract research organizations, will gather at the Hemophilia Drug Development conference in Boston on Aug. 14 through 16.

The meeting is designed to be an industry-dedicated networking and discussion forum, and many of the major experts in hemophilia — pharmaceutical and academic — will participate. About 80 percent of all attendees are expected to be industry representatives, followed closely by academic researchers (8 percent) and "solution and service providers" (9 percent).

Participants will have the opportunity to gain new insights into the long-term impact of new and potentially revolutionary extended half-life products, non-factor products, and gene therapies that are being developed to treat hemophilia.

Discussions will also focus on strategies to overcome the hurdles in the development of, and marketing access to, new hemophilia treatments, facilitating their translation into the clinic.

Several key aspects of drug development will be addressed, including:

- Optimizing patient-centric approaches to ensure clinical trial success;
- Improving the recognition of underlying biological mechanisms involved in next-generation therapies;
- Better understanding of patient motivations to adopt novel therapies;
- Exploring how to improve clinical trial design to obtain clinically meaningful results in an era of new drug development;
- Discussing, in-depth, reimbursement models that work to ensure effective market access.

The summit will have 21 expert speakers, including Robert Peters, senior vice president of research at Bioverativ; Henry Mead, global medical director of hematology at CSL Behring; Mark Skinner, president and CEO of Institute for Policy Advancement; Alison Schecter, global program head of rare diseases at Sanofi Genzyme; and Howard Levy, chief medical officer of Catalyst Biosciences.

Pharmaceutical companies including Spark Therapeutics, Novo Nordisk, uniQure, Bayer, and Shire will also be represented at the conference.

Such discussions are highly relevant to hemophilia patients and caregivers, said Michelle Rice, senior vice president for external affairs with the National Hemophilia Foundation. That's because those touched by hemophilia are both interested and motivated to embrace new and promising treatments that impact everyday life.

"These new therapies have the potential to greatly improve the quality of life for those eligible to utilize them," said Rice, who will speak on a panel looking at ways of demonstrating treatment benefits while linking them to costs.

"[I]magine being a patient with severe hemophilia and an inhibitor and having to infuse yourself 15–30 times a month; im-agine how that impacts your ability to engage in work, school or social activities, Rice said. "Now imagine being able to infuse subcutaneously once a week or even once a month…what an amazing sense of freedom!"

But, she continued, "This is a community that has experienced more than their share of issues related to product safety. … Advocacy efforts" are, for this reason, crucial to ensuring access and acceptance.

Participants will also have the opportunity to register for two pre-conference workshops set for Aug. 14.

Workshop A will be led by Liselotte Jansson, chief research officer at Apitope, and will be address immune reactions against hemophilia therapies and strategies to overcome them.

Workshop B will be led by Irina Matytsina, international medical director of global development at Novo Nordisk, and will discuss clinical trial design and strategies to optimize their outcome.

Early registration discounts are offered through July 13; more information is available here. Pricing differs according to type of registration (industry or academic, for instance), and savings of up to $600 off regular prices are possible.

Event organizers say attendees will have the opportunity be part of "cross-industry discussions" that could pave the way for advancing "innovative hemophilia therapies that will transform the lives of patients."

The meeting's venue is The Westin Boston Waterfront, 425 Summer St., Boston. Registration details can be found online, by email at info@hansonwade.com , or by calling +1-617-455-4188.

Source: https://hemophilianewstoday.com/2018/07/06/morphological-drug-development-summit-takes-aim-at-new-treatment-hurdles/

The team at the Midland Regional Children's Workshop

The Year Ahead...

August 31 - September 2

- **Central Winter Escape**
  Kennedy Park, Napier

September 14 - 16

- **Women's Wellness Weekend**
  Copthorne Oriental Bay, Wellington

October 20 & 21

- **HFNZ AGM and Regional Office-holder Workshops**
  Copthorne Oriental Bay, Wellington

Jan 25 - 28, 2019

- **HFNZ National Family Camp**
  Woodend Christian Camp, Woodend, North Canterbury

November 8 - 10, 2019

- **HFNZ 60th Anniversary Celebrations**
  Wellington

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