December 2018
Magazine of the Haemophilia Foundation of New Zealand. Volume 46 Number 3

Bloodline

HFNZ in 2018
The year in pictures

Haemophilia Foundation of New Zealand Inc.
HFNZ

Welcome to our new CEO, Sue Ellis «

AGM & Training Weekend «

Bloodline

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The H Word

In the lead up to summer, it's really heating up at HFNZ!

I am absolutely delighted to welcome Sue Ellis to the Haemophilia Foundation. Sue started with us in mid-November as our new CEO. Sue brings vast experience to us coming from the health and disability and community sectors. It's hard to sum up in one sentence. You can read more about Sue in this issue. Sue has already managed to attend every regional Christmas event this year and is truly hitting the ground running!

We have also recruited a new outreach worker for the Northern region. The region's former outreach worker, Nicky of the North, is moving to the Midland region - so we now benefit from her enthusiasm and dedication to our community in two regions! On December 5 Amber Maihi starts with us in the Northern region. We are looking forward to welcoming her to the Foundation.

Following the AGM in November, we are now looking at national activities for 2019. Some of you will have seen that we originally planned a family camp in January. From the responses we received, April 13-15 is now pencilled in - so look out for more information. We also plan to hold a youth camp around the July school holidays. To round off 2019, we are going to hold our 60th celebrations on November 8 & 9 in Wellington. I know there are many activities planned in the regions, too; 2019 is filling up fast.

On the treatment front, PHARMAC is currently assessing proposals from industry, and will soon announce the preferred providers. HFNZ's preference is longer-acting FVIII and FIX available for all, a product switch if it is for longer-acting, but enough stock of other therapies when there are sound clinical reasons not to switch products. We also have our eyes on novel therapies such as emicizumab (trading as Hemlibra) and the promise of gene therapy.

2019 will not be without its funding challenges, with the Ministry of Health contract to support all people with hepatitis C and haemophilia coming to an end. We will need to secure new partnerships and opportunities. The proportion of government funding we receive is negligible, and so we are incredibly grateful to the public, community organisations, trusts, benefactors, and industry for enabling us to continue providing services to people with bleeding disorders that cannot be found anywhere else in the system.

We have an exciting year coming up, and change will bring new opportunities for all our members.

I do hope that you all have the opportunity to get some much-needed rest over the summer and time with your loved ones.

Deon York
HFNZ President

Introducing... Our new staff

Ko Takitimu te Maunga
Ko Waiau te Awa
Ko Murihiku te Marae
Ko Te Au te Whanau
Ko Ngati Mamoe te Hapu
Ko Ngai Tahu te Iwi

Sue Ellis

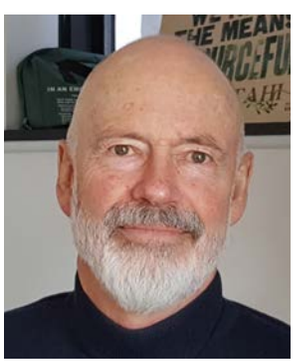
Chief Executive RN; B.Ed; PG BusAdmin; MBS

Commencing with HFNZ on 14 November 2018, Sue comes to the position of CEO with a broad range of experience in health and education. She has worked for many years in government and non-government organisations in many roles, including clinical manager, policy analyst, advisor, project manager, and strategic policy manager.

Sue trained in mental health nursing and taught for a number of years in the graduate nursing programmes. She later worked as a nurse consultant before moving to the Ministry of Health as a policy analyst, and then as a senior advisor with the Mental Health Commission. For the last few years, after setting up a consultancy business in health, education and research Sue has been providing consultancy services to a wide range of health organisations.

Sue lives by the beach north of Levin with her partner, their dog, and two cats.

"I am so excited to have this opportunity to be working with such a fabulous group of enthusiastic, committed, and inspiring bunch of people. A whakatauki that underpins my belief in the strength of HFNZ is He waka kōhuia kāhore e tukutukua nga mimira, A canoe that is interlaced will not become separated at the bow. In unity there is strength".



Ross Paterson

Southern Outreach Worker

Commencing in August 2018, Ross has been working as the Southern Outreach Worker for Haemophilia New Zealand. Ross is available to provide education, support and advocacy to families and individuals. He lives in North Canterbury, close to Christchurch.

Ross is a qualified social worker with extensive experience in diverse roles such as Probation Officer, Victims' Advisor at the Court and Career Counsellor. In the latter role he worked alongside individuals who were unable to return to their previous type of employment and needed to find a new employment direction in their life.

Ross is motivated by working alongside others and helping them to find solutions to issues in their lives. He enjoys working with people across the age spectrum and in all walks of life.

Away from work Ross is an outdoors person who enjoys growing food free of pesticides and insecticides and enjoying tramps and walks in the bush / hills. He still sees magic in a seedling popping up through the ground! He values family time and catching up with friends. Reading and listening to music are other keen interests.

Farewell from Richard

BY RICHARD CHAMBERS

HFNZ is an organisation of people; caring passionate people. Too often, we find ourselves living in a world where some people somehow seem to be forgotten about, but not in the bleeding disorder community, where everything always comes back to the simple question... What positive difference will this make to people impacted by a bleeding disorder?

This question is what drives the foundation. When I have worked with the council, with members, with staff, and in our discussions with funders and supporters, it has always been top of the mind. Sometimes we will have different opinions about how to get there, but never about where we want to get to.

HFNZ is one of the few patient-owned organisations that still provides quality low or no cost national education workshops and camps to their membership, supports members in need, and has access to an educational endowment fund to support members in their educational choices.

The foundation is embarking on setting a new path forward for the next ten years, it is important your voice is heard; a person with a bleeding disorder (including carriers), a parent, a brother, a sister, a spouse or partner, old or young it doesn't matter, make sure your voice is heard. Your opinions and views matter.

Now is another pivotal moment in HFNZ history. New and better treatment options are becoming available and must be accessible in New Zealand. By moving head office to Wellington, and appointing a new Wellington based CEO and administrator, HFNZ is placing itself upfront and close to the political and pharmaceutical decision-makers.

I am honoured to have been part of the HFNZ for over four years. I hope the work the staff team did to enhance HFNZ's capability and to adapt to the changing needs of the community has set a strong platform for the next stage of HFNZ's education, advocacy, and support journey. Together you will improve the lives and long-term impacts for people impacted by bleeding disorders. Ki kaha. Stay strong.

Thank you for enabling me to be part of your lives and journey.

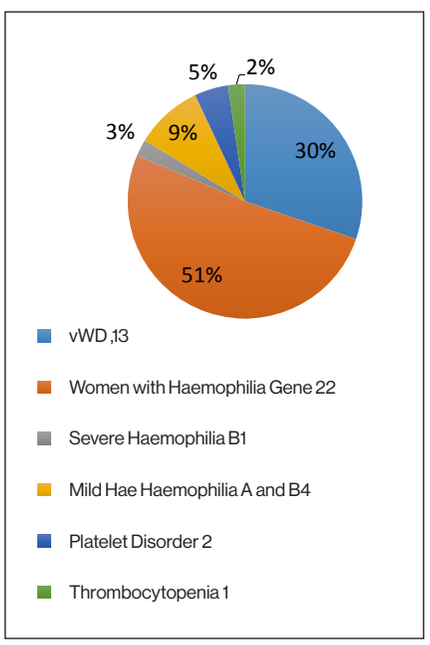
HFNZ Women's Weekend 2018

BY LYNNE CAMPBELL

In recent years, there has been increased recognition of the unique issues faced by women with inherited bleeding disorders.

We held this two-day event for Women with inherited bleeding disorders aged from 18 years and upwards at the Copthorne Oriental Bay in Wellington over October 15 and 16 2018. Although we held a Young Women's Weekend in 2016, it has been five years since women over 28 have had the opportunity to attend a Women's Workshop.

Participants: 43 Attendees of all ages, (aged 18-approx. 80) and three Outreach Staff.



Aims and Objectives

• To provide information and education.

• To empower participants to understand and work through the issues associated with their condition.

• To develop a sense of community within the group.

Programme Day 1

9:00-10:00 Arrive, Morning meet-&-greet, Warm up.

10:45 Morning tea

11:00 Keynote Speaker: Dr Claire McLintock + Q & A with Claire

12:30 Lunch

1:45 Sewing table

3:00 Afternoon tea

3:15 Let's talk about sex

• Health Professional - B.J.

• Reproduction - Hannah

4:30 Sewing table / buy and sell for Nepalese Foundation

5:30 Social time

7:00 Dinner Blind auction

Programme Day 2

8:30 Morning song

9:00 Andie Dunn: Colour & Style Consultant

10:30 Morning tea

11:00 Women and Bleeding: personal experiences and Expert Panel Discussion with a woman representing each of obligate/carrier with the haemophilia gene, vWD and no Family History.

12:15 Lunch

1:00 Giving back to HFNZ (panel discussion about involvement from three who have had a close association with HFNZ from Committee level through to Council level.

2:15 Afternoon tea

2:30 Weekend windup, closing remarks

3:00 Leave for airport

While education was the primary focus of the programme, recreational activities and time for bonding was included in the programme and appreciated.

Dr Claire McLintock's presentation "Women and Girls with Bleeding Disorders" was the outstanding favourite educational session. In their evaluation, several attendees commented that Claire's talk had changed their thinking and understanding around how haemophilia affects women.

Fog at Hamilton airport unfortunately resulted in the Hamilton group missing all of day 1 and some other flights were to a lesser extent delayed. In their evaluations, many participants requested that in future, everyone arrive the night before so that there were no delays in starting the main programme. In addition, some participants requested that an offsite social activity be included for future Women's Workshops.

All participants enjoyed hearing the personal stories of others, catching up with old friends and meeting new members. Participants left the event feeling more informed and empowered.

2018 AGM & Training Weekend

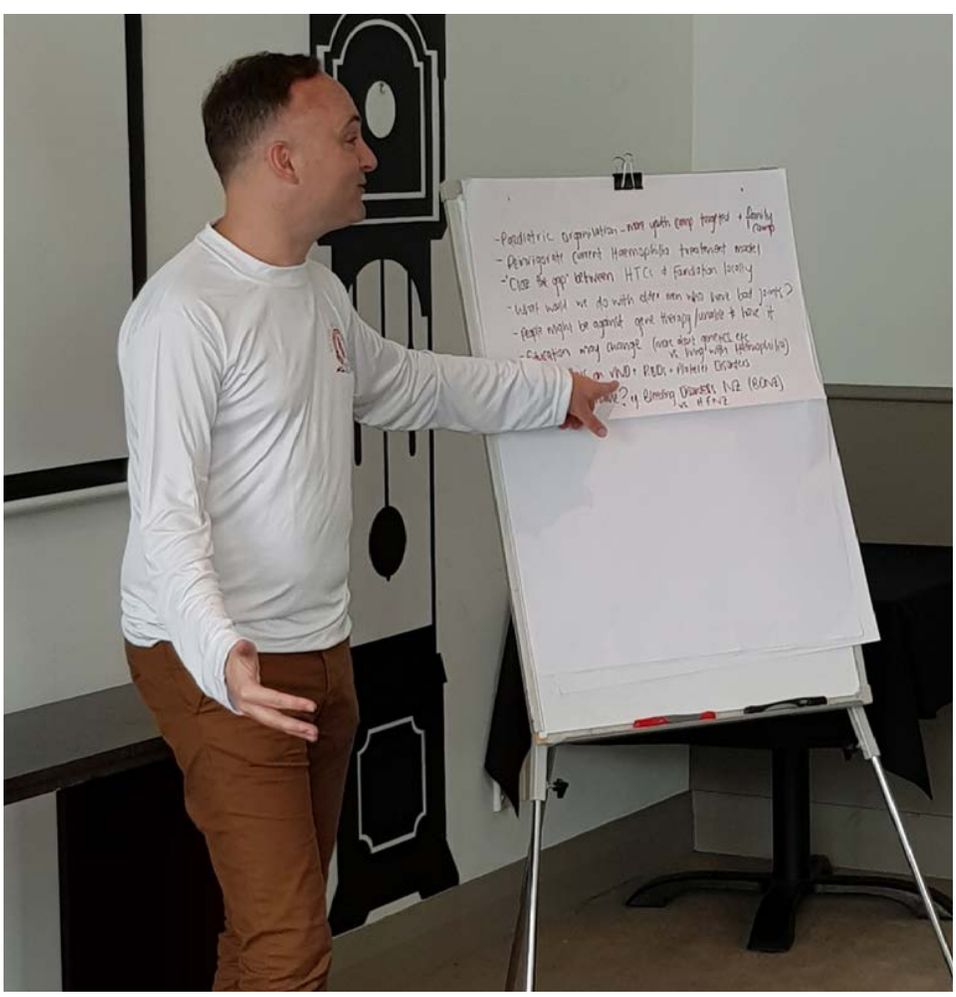
Over the weekend of November 3 & 4, at the Copthorne Oriental Bay in Wellington, HFNZ held its AGM, and ran training for members who had stepped up to regional and group committee roles. We also had the opportunity to farewell our outgoing CEO, Richard Chambers, and to welcome our new CEO, Sue Ellis.

The weekend started with our last National Council meeting for 2018. We discussed plans for 2019 and spent time with the staff.

We started the AGM by remembering those who had gone before us, and noting the passing of Daphne Walsh, Graham Waring, and Neville Findlay in the year since the last AGM. Catriona Gordon lit the memorial candle, which stayed alight throughout the meeting.

At the AGM we finalised the National Council for the next year, as well as the regional representatives. The executive remains Deon York, president; Catriona Gordon, vice-president; Karl Archibald, vice-president; and Hemirau Waretini, treasurer. Joining the executive on council are Tineke Maoate, midland; Stephanie Coulman, central; Theresa Stevens, southern; Te Whainoa Te Wiata, Piritoto; Courtney Stevens, youth; co-opted member Steve Waring of KiwiFirst; and our new CEO, Sue Ellis.

Next was awards time. HFNZ always likes to recognise the great things our people do through the year. This year we awarded the McKay Trophy for regional or group activities, and the Elizabeth Berry Exercise Cup. We were very pleased to award the McKay Trophy to the Piritoto group, for the great strides they've taken in developing focused and valuable noho throughout the year. Connor Jamieson was the worthy winner of the Elizabeth Berry Cup. Connor has been swimming at a very high level during the last year, and has committed himself to frequent training, setting a fine example for us all.



The AGM wound up by recognising staff and members who had moved on to new roles through the year. Top of the list was former CEO Richard Chambers. Richard had been with HFNZ for over four years, and had worked hard for all HFNZ members during his tenure. He has demonstrated sound financial management and a genuine care for all our people.

Also acknowledged were former staff members Karen Melville, Josiane McGregor, Ali Mitchell, and Joy Barrett, as well as former National Council member and Midland Chair Linda Mellsop-Anderson. HFNZ appreciate the contribution of all of these friends of HFNZ, and wish them well in the future.

The remainder of the weekend was given over to training and strategic planning.

President Deon York started by talking about where we'd come from as a foundation, and what challenges we had ahead of us. He highlighted the need for inclusiveness and member-involvement, and recognised the importance of regularly reviewing goals and processes so that we remain effective as an organisation.

Deon then led us to explore the types of behaviours that might be expected to render us ineffective. The goal was to identify ways that we may be inhibiting our own development and effectiveness. This was a very interesting exercise, which produced some fascinating results to feed into the next exercise, where we looked at key ways to make us more effective as a foundation. The participants enjoyed both these sessions, and came up with a whole lot of really great ideas.

That evening a large group of members and staff headed out to enjoy a cheap meal. Everyone had a great time, and appreciated the time to connect with each other in a more informal setting.

The next morning was the final session, this time devoted to planning for the year ahead. We looked at what each region and group has planned, as well as the national events that are due over the next 12 months, to try to put together some sort of calendar of events. We ended up with quite a full programme for the year, and figured out ways to keep each other informed and up-to-date.

Overall this was a valuable weekend, which allowed us to move into 2019 with some structure and confidence. It was fantastic to be able to recognise the contribution of members and staff across the year, and to welcome newcomers into the fold. Best of all, it waszzzz once again, great to be able to have members and staff together in one place to reconnect and reaffirm our commitment to HFNZ.

HFNZ in 2018
The year in pictures.

Top: The knitters.

Middle left: McKay Trophy Winners - Piritoto.

Middle right: Joy at her farewell.

Bottom: Ashley & Deon talk youth twinning at congress.

Top: At Joy's farewell.

Middle: Babies welcome at Women's Weekend

Bottom: Dinner at congress.

Radioactive Synovectomy as a treatment for Synovitis

BY HELEN DIXON

What is Synovitis?

Synovitis is the inflammation and thickening of the Synovium. The Synovium is the thin layer of cells that lines the joint. It is responsible for "cleaning up" blood from the joint after a joint bleed. The iron within the blood acts as an irritant to the synovium causing it to become inflamed and to thicken – SYNOVITIS

This thickened, inflamed lining is now more likely to be trapped or damaged during joint movement. This will lead to more joint bleeds starting a cycle of bleed and rebleed.

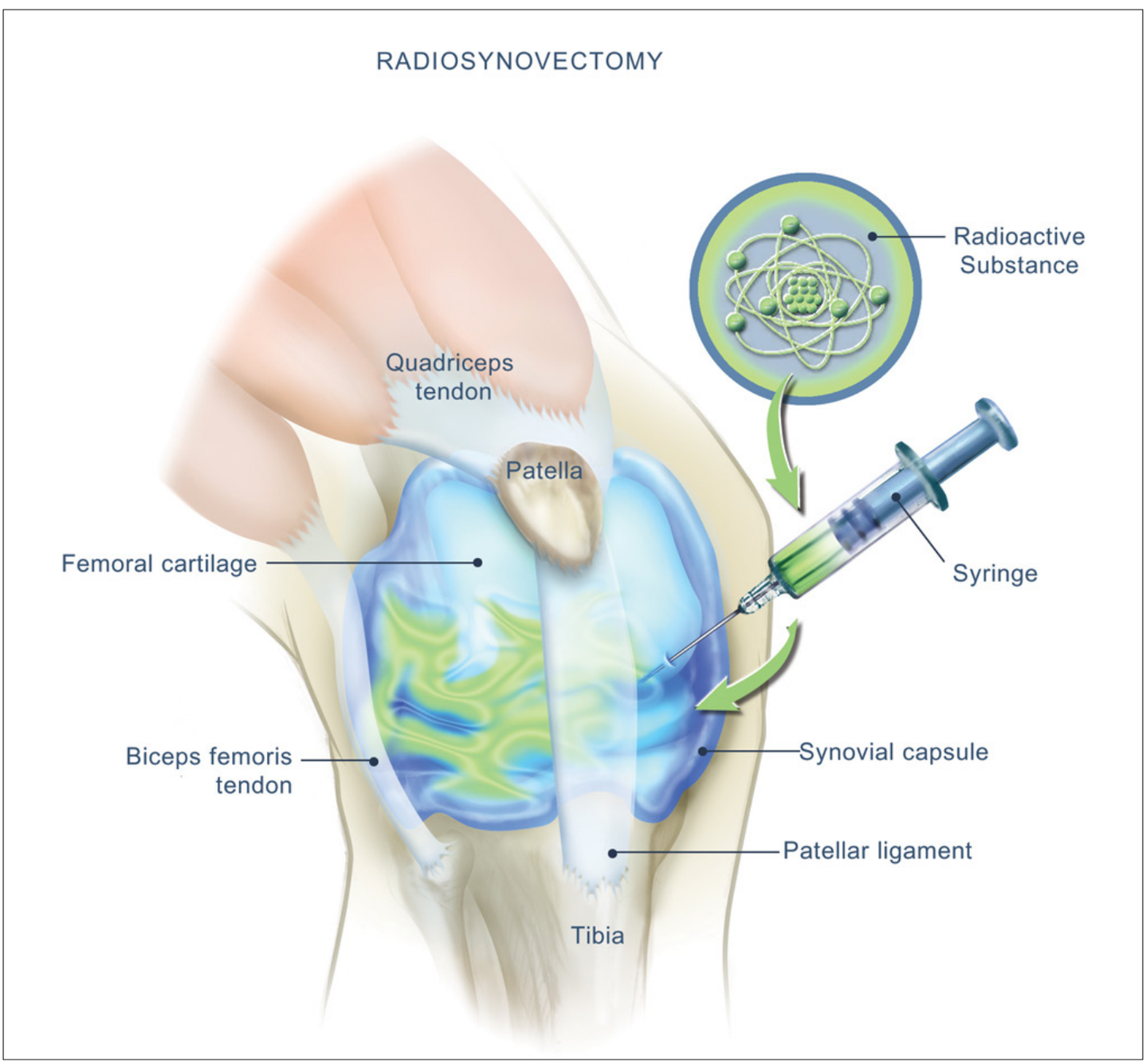
How do I know if I have Synovitis?

The joint will be swollen and usually hot (but not always), this will be more persistent despite factor replacement therapy. Often range of motion in the joint is only slightly reduced.

Synovitis can be diagnosed by ultrasound or MRI scanning and this should be done through your Haemophilia treatment centre.

How does Radioactive Synovectomy Work?

The radioactive isotope is injected directly into the joint. This has the effect of reducing the number and size of synovial cells and villi (protrusions or dangling strands of the synovium). It also reduces



the blood flow to the synovium. This helps to reduce the synovitis significantly. Consequently, the synovium is less likely to be injured during joint movement and the number of joint bleeds reduces. This in turn will help to reduce the speed of haemophilic arthropathy (arthritis) development.

A positive response and reduction in pain has been documented in 75% of patients.

Is Radioactive Synovectomy safe?

The radiation dose that the patient is exposed to is less than the recommended annual dose. A recent longitudinal study of 2412 patients who underwent radioactive Synovectomy showed no increased incidence of neoplasm (cancer). There have also been no confirmed reports of malignancy (cancer) as a result of radioactive Synovectomy in children.

What is involved in a Radioactive Synovectomy?

The Radioactive synovectomy procedure involves an intra-articular injection, a needle directly in to the joint space where the synovitis is seen. You will have a local anaesthetic injection (using the same needle) into the joint first to help reduce the pain followed by the Ytrium (radioactive isotope) injection. It may take up to 2 weeks before any relief occurs after an injection of Yttrium. Initially the joint may in fact be more painful. Some patients describe it feeling like a bleed, as this fluid is in the joint. This shouldn't last more than a few days.

Factor replacement therapy is administered directly before the Ytrium Injection at the dose recommended by your HTC.

After the injection the joint is put in a splint to stop it from moving and to help keep the Yttrium in the required place for 48 hours. Make sure that you wear clothing that allows easy access to the joint being treated and that will fit over the immobilising splint after the procedure. You can eat and drink as normal.

Local anaesthetic is injected below the skin of the joint where the Yttrium is to be injected. This may sting for 10 to 20 seconds. Once the skin is numb, a needle is inserted into the joint. Sometimes an ultrasound is used to position the needle into the correct position.

Once the needle is in the joint, the radioactive isotope (Yttrium) is injected into the joint, followed by an injection of steroids. The needle is removed, a firm bandage applied, and a splint fitted to keep the joint immobile.

The injected limb must be kept immobile for the first 48 hours after the injection. You will be placed in a splint to assist with this. If you have had an injection in your knee, this is the reason for you to stay overnight in the unit. If you have had a knee or ankle injection you will need to use crutches for this time as well and keep the joint non weight bearing. The splint, bandages and dressings may be removed after 48 hours. The bandages dressing must be placed in a plastic bag, tied up and disposed of in the household rubbish. Wash your hands after handling the bandages and dressings.

Avoid any strenuous activity involving the affected joint for 2 weeks.

A Physiotherapy appointment will be arranged following the injection.

Make sure you know the Factor replacement therapy regime that has been recommended for you and please record this in your treatment records.

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Image source: https://www.rarebleedingdisorders.com/bleeding-disorders/congenital-hemophillia.html

Female Factors: A new young-women's resource

Haemophilia Foundation Australia has released Female Factors, an innovative new resource for young women and teenage girls.

This fantastic magazine has found its way across the Tasman, and has been very much impressing HFNZ people. At the recent AGM & training weekend we had many people commenting on what a valuable resource this is, and how amazing it is that, after all these years, women with bleeding disorders are getting some well-deserved attention.

Bloodline reached out to HFA to get the low-down on Female Factors. Here's what they had to say...

We developed Female Factors to answer young Australian women's questions about how bleeding disorders affect females – but in a magazine style that is fresh and engaging. There are personal stories, quotes, and tips.

HFA worked with haemophilia and gynaecology experts to put together easy-to-read information for young women, which focusses on what they really want to know.

Feedback has been very positive.

'I love the design and layout. The look and feel is great, very welcoming.'

'This is a fantastic resource and covers so many areas.'

The booklet has explanations about heavy periods and other bleeding symptoms in females. It looks at haemophilia - and why girls with haemophilia have different bleeding patterns to their father or brothers - von Willebrand disease (vWD), rare clotting factor deficiencies, and inherited platelet disorders. It also covers other key issues for young women, including inheritance, diagnosis/testing, treatment and support, and tips for self-advocacy from other women. It also includes FAQs such as what's 'normal' and what is not normal, and gives frank but reassuring answers to some of the questions that worry young women.

Many people were involved in developing Female Factors. Young women and their parents gave thoughts on the content and reviewed the text. HFA staff looked at young women's concerns in social media and prepared FAQs. Haemophilia health professionals and other experts suggested what topics to cover, reviewed it thoroughly and wrote new material to answer questions. The text was rewritten for a young female audience by a health educator. HFA and a focus group of young women worked with a designer so that the design was young and attractive to read.

Special thanks go to the young Australian women affected by bleeding disorders who contributed their personal stories and tips and focus-tested the design!

How to access it

Female Factors is available in multiple formats

• On the HFA website www.haemophilia.org.au under PUBLICATIONS

• On Factored In www.factoredin.org.au under INFO > GIRLS

• You can read the entire booklet online as a magazine in ISSUU, download it, or download specific sections

To find out more about Female Factors, contact HFA:

E: hfaust@haemophilia.org.au

HFNZ Member Updates

Medic Alert alternatives

Medic alert is a company that maintains a database of paid up members, and records their medical history and details any allergies and/or disorders. To belong you need to pay an annual fee. For that, you get a bracelet with all your details on it, and those details stored electronically for access when required.

This system has worked reasonably well for many years.

HFNZ members have told us that they have had increasing difficulty with the way their details are managed, and with the administrative and monetary burden that goes alongside it. Some members have tried to withdraw from Medic Alert, and have found that there are a number of hoops to jump through, and that there appears to be a cost for withdrawal.

On top of that, we've had advice that first responders in NZ tend not to use the system. Rather, they prefer to be able to access a patient's details by using their national health index (NHI) number. These days, first responders have instant access to NHI data via the Internet from the ambulance. That means, as long as they know your NHI number they can very quickly know exactly what your issues are, and can give the most appropriate treatment. However, what happens if you're unconscious?

With that in mind, there are a number of alternatives that will work just as well, if not better than Medic Alert, for a much lower cost.

Most simply, you can just buy any bracelet, and have your NHI number engraved on it. The issue with that is that first responders may not recognise the bracelet for what it is.

A couple of our members have sourced more targeted alternatives:

One of these options is www.mediband.co.nz. This company has a database service, but they also sell a wide range of different types of bracelets, without the need to sign up to their service. Bracelets range from pre-printed silicone bands, to stainless steel chains. They also have a variety of add-on badges for their bracelets that highlight different medical issues. The minimum you will need is a bracelet with a plate that has your NHI number engraved on it.

The other is www.onelifeid.com. This is a UK based company, which also has a wide variety of bracelet styles. Some of these are very styley indeed! The price is higher than the mediband, but there are some very nice options. Again, you get to choose what you have engraved, but as a minimum, your NHI number is recommended.

Celebrex change

Many HFNZ members have used Celebrex over the years. Celebrex is a nonsteroidal anti-inflammatory drug (NSAID). It works by reducing hormones that cause inflammation and pain in the body. Celebrex is used to treat pain or inflammation caused by many conditions including haemophilia.

In December 2016, after a round of consultation, Pharmac notified that they had awarded sole-supplier status for this class of medication to Pfizer. Because Pfizer were the producers of Celebrex, the biggest change here is the change of medication name. The brand name is now Celecoxib Pfizer.

Many of our people, including staff, had no idea about the change. One of our members went to fill his usual prescription, only to be told that he would have to pay $1200 for that medication! Luckily, he had a good relationship with the pharmacy, and they quickly fixed the error.

It's important to know that HFNZ people who are prescribed medications like Celecoxib Pfizer access them under special authority granted by the Ministry of Health. Special authority can provide:

- access to subsidy
- increased subsidy, or
- waive certain restrictions otherwise present on the Community Pharmaceutical.

When you're next picking up medication for pain, if the prescription is for Celecoxib Pfizer, did you know that it's subsidised? That means you should only pay the $5 prescription fee. When your doctor prescribes this medication, they should have a special authority number. By using this, they will access the subsidy so you don't have to pay full price. It's a good idea to check this number with your doctor.

If you think that your pharmacy has got it wrong, make sure you tell them that you are able to access the medication under special authority, and quote your number.

You can find out more about Celecoxib Pfizer here: https://www.medsafe.govt.nz/Consumers/cmi/c/celebrex.pdf

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 Report

By Stephanie Coulman

Our winter escape to Napier was a fun weekend for all staying at the Top Ten in Napier, in late August. We were lucky to have Haemophilia Nurse Specialist BJ Ramsay and Haemophilia Physiotherapist Helen Dixon as guest speakers. BJ spoke about new therapies; there is a lot happening in this space particularly longer lasting factor treatments and BJ was able to share learnings fresh from the 2018 World Congress in Glasgow. Helen spoke about Wellington Hospital's new ultrasound programme and rheumatology access.

Between the two of them, they are a formidable source of knowledge and support, and we are lucky to have them on our side.

We held our AGM at camp and there were no changes to the committee. The central region continues to be ably led by Chair: Blair Wightman, Treasurer Ross Gordon, Secretary Ashley Taylor-Fowlie, and National Council Delegate Stephanie Coulman.

Our happy campers enjoyed a soak at Ocean Spa and experienced the marine wonders of our National Aquarium, the two pearls of Napier's Marine Parade.

Our Christmas event on 24 November was well attended with a great turnout, very pleasing for the organisers! We had lunch and enjoyed a visit at Southward Car Museum in Otaihanga, home to a collection of over 400 vehicles, as well as three aircraft. This privately owned collection is a delightful peek into the vehicles of yesteryear.

We are looking forward to a repeat of our successful men's fishing trip, on 10 March 2019 (weather permitting). This will be for the blokes so look out for your invitation in the New Year.

Until then, on behalf of the central region committee, we wish you a very Happy Christmas and health and happiness for 2019.

Midland Report

By Tineke Maoate

A big thank you to everyone attended the Christmas Rainbow's End event in Auckland. It was an awesome day. For all of those that couldn't make it I wish you a merry Christmas and a happy new year.

We are in the process of planning an event in Taupo at the beginning of the year, which will include a summer bbq and some water fun. If anybody has some ideas for activities for Midland to do so we can get together, please let me know. We are working on putting a great family camp together too.

Keep safe and I look forward to seeing everyone at our next event. Please feel free to contact Nicky or me if you need anything, or if you have some ideas.

Southern Report

By Zac Porter

Kia ora all.

The Southern region has been busy and is looking forward to our upcoming Christmas get-together, which we're holding at Willowbank on December 9. This should be a great time to re-connect with our community, check out the animals, and share some kai.

In addition to this, there is a youth event in the pipeline, which the Southern youth should hear about soon. We hope to run an event for the adults too next year, which may involve a fishing trip, watch this space.

In the meantime, we hope everyone has an excellent Christmas and New Year.

From top.

1. Midland youth bowling 2018.

2. Zac & Santa at the Southern Xmas do 2018.

3. Some central region members enjoying the Southward Car Museum, including new CEO Sue Ellis (2nd right).

4. Happy campers in Napier, August 2018

In Memory of Neville Findlay

BY ROSS PATERSON

It is with sadness that we note the passing of Neville Findlay, one of life's characters and a member of the foundation for the last decade or so, who passed away in the early hours of 20 October.

Members of the Foundation may remember Neville as he was at the last Camp that he attended several years ago. By this time he had limited mobility and would install himself in his mobility scooter for the day. Despite this, Neville acted like a honeypot to bees, where people would regularly go to spend time with him, to be charmed by his dry wit, twinkle in his eye and readiness to engage in conversation with anyone.

Many may not be aware that Neville trained as a dental technician and primarily worked in this role, although he also had stints managing the tuck shop at the Papanui Club and made glass eyes at Burwood Hospital. He played the drums in a band in his younger days, golf regularly until twenty years ago, and was a cricket umpire while in his thirties and forties. In later years as a bachelor, having a quite beer, reading, and watching TV were favourite pastimes.

News from around the world

Initial treatment with emicizumab improves factor VIII tolerance in hemophilia A

BY JULIAA ERSNT MS

Immune tolerance induction with standard or extended half-life recombinant factor VIII after initial treatment with emicizumab appears safe and effective in pediatric patients with hemophilia A and active inhibitors, according to findings presented at the ASH Annual Meeting and Exposition.

"While novel non-factor therapies significantly reduce bleeding symptoms in patients with hemophilia A and inhibitors, the absence of [factor] VIII tolerance remains unchanged. Additionally, there are concerns regarding the hemostatic efficacy and safety of bypassing agents necessary for the management of breakthrough bleeds in patients with inhibitors on these novel therapies," the researchers wrote. "Immune tolerance induction remains the primary method for eradicating inhibitors and restoring the hemostatic response to [factor] VIII."

Glaivy M. Batsuli, MD, assistant professor in the division of hematology and oncology at Emory University and pediatric hematologist/oncologist at Aflac Cancer and Blood Disorders Center in Atlanta, and colleagues examined this approach, known as the Atlanta Protocol, in eight patients aged 13 months to 11 years with hemophilia A and an active inhibitor (0.6 BU/mL). All but one patient had severe hemophilia and most patients (n = 5) had at least one prior attempt at immune tolerance induction. Three patients needed central venous access for factor VIII infusions during this process.

Immune tolerance induction began after four weekly loading doses with emicizumab and standard or extended half-life recombinant or plasma-derived factor VIII at 100 units/kg administered three times per week. The researchers selected a factor product based on the agent used in prior immune tolerance induction attempts; a standard half-life, third-generation recombinant factor VIII product was used if the patient had not attempted immune tolerance induction before.

Pharmacokinetics measured during immune tolerance induction included estimated factor VIII half-life, expected factor VIII incremental recovery and inhibitor titers. These measurements were taken with chromogenic-based assays that used bovine reagents. The researchers also monitored bleeding symptoms and treatment regimens for bleeds/procedures.

The researchers reported "historical" peak inhibitor titers, ranging from 2 to 198 BU/mL, among all patients in the study. Historical was defined as the highest inhibitor titer prior to the start of the immune tolerance induction done in the study.

CEMA: Patient Registries Can Replace Clinical Trials for Certain Hemophilia MAAs

BY ZACHARY BRENNAN

For companies developing marketing authorization applications (MAAs) for recombinant and human plasma-derived factor VIII and factor IX hemophilia medicines in previously untreated patients (PUPs), the European Medicines Agency (EMA) announced Friday that data should be collected from patient registries rather than from small clinical trials.

Revised guidance from EMA spelling out the changes notes the aim is to optimize the use of these registries and provide parameters for core data sets that should be collected.

"The new approach described in the hemophilia guidelines waiving the requirement for a clinical trial in previously untreated patients was discussed at an EMA workshop on hemophilia registries in July 2015 where the use of registries in hemophilia was explored," the agency explained.

The revised guideline notes that discussions at the workshop centered around how the number of suitable patients, especially PUPs, to be enrolled in clinical trials is problematic.

"Hence, the conduct of sufficiently informative clinical trials in PUPs to estimate important characteristics of single products is considered difficult. Following a public consultation in 2017, a second workshop on hemophilia registries was held on 8 June 2018 which aimed at defining the requirements for practical implementation using existing registries to support post-authorisation observational studies of hemophilia medicines."

The workshop discussed recommendations on the appropriate governance of registries, patient consent, data collection, data quality and data sharing, and interoperability between different registries.

"Therefore the obligation to perform clinical trials in PUPs for marketing authorization purposes has been deleted," the guideline says.

EMA is also offering a parameter set for hemophilia registries in the guideline, seeking information on demographic data, anamnestic information, hemophilia treatment information, inhibitor information and other relevant information on concomitant events.

The revised guideline for hemophilia medicines for factor VIII deficiency was published in July 2018, and the revised guideline addressing medicines for factor IX deficiency is published today.

The public consultation on the revised factor IX guideline ends 30 June 2019.

Source: **https://www.raps.org/news-and-articles/news-articles/2018/11/ema-patient-registries-can-replace-clinical-trial**

Potential Gene Therapy for Hemophilia A, AMT-180, May Treat Patients Regardless of Inhibitor Status, uniQure Says

BY JOSE MARQUES LOPES, PHD

A gene therapy from uniQure in early testing, called AMT-180, has the the potential to treat all hemophilia A patients, including those with inhibitors, according to the company.

Hemophilia A is caused by missing or defective factor VIII (FVIII), a clotting protein. About 30% of patients with severe hemophilia A develop inhibitors, or antibodies, that neutralize FVIII activity.

AMT-180 is designed for one-time intravenous (IV) delivery and uses a type of viral vector called adeno-associated virus 5 (AAV5). The potential therapy contains a modified factor IX gene known as Super9, which has shown an ability to bypass inhibitors to FVIII in preclinical studies, suggesting it may be beneficial for a patient population previously excluded from gene therapy approaches.

A proof-of-concept study in mice also demonstrated that Super9 had clinically relevant activity mimicking FVIII, and was not associated with exaggerated blood clotting.

In primates, a single AMT-180 dose led to expression levels corresponding to FVIII activity likely to be clinically relevant in hemophilia A patients with or without inhibitors, the company said. These findings were supported by Super9-induced activation of the blood clotting protein thrombin in FVIII-depleted human blood with or without inhibitors.

Results suggest long-term prevention of bleeds in hemophilia A patients treated with AMT-180, the company said.

uniQure also recently issued a company update on ongoing patient enrollment in its open-label Phase 3 trial (NCT03569891) of a potential gene therapy for hemophilia B, called AMT-061.

And it announced that three patients taking part in a Phase 2b study (NCT03489291) of AMT-061, which is also still enrolling eligible patients, have already been treated.

At its Research & Development (R&D) Day, held on Nov. 19 in New York City, uniQure also announced the development of other one-time AAV approaches to possibly treat Fabry disease and spinocerebellar ataxia type 3 (SCA3).

For Fabry disease, uniQure is advancing AMT-190. This therapy is intended to evade anti-GLA protein antibodies found in most male patients, so as to be a more effective and longer-lasting therapy.

For SCA3, also known as Machado-

Joseph disease, uniQure is developing AMT-150, which uses the company's new miQURE technology to halt ataxia — a lack of voluntary coordination of muscle movements — in patients with early disease symptoms.

"We are very proud of the progress the Company has made to deliver extensive preclinical data for these new gene therapy programs that expand our pipeline and further validate uniQure's potential best-in-class vector delivery platform," Sander van Deventer, MD, PhD, uniQure's chief scientific officer, said in a press release.

The new gene therapy candidates represent a step forward "towards uniQure's goal of delivering transformational medicine to patients suffering from genetic diseases. We look forward to advancing these programs closer to the clinic in 2019," van Deventer added.

uniQure also presented advancements in technology and manufacturing, topped by miQURE, a technology platform designed to safely degrade disease-causing genes. miQURE is intended to induce long-lasting gene silencing of the entire target organ. Improved tissue specificity and more pronounced gene expression lowering were seen in preclinical studies. Besides AMT-150, miQURE has been incorporated into AMT-130, a Huntington's disease treatment candidate.

The company also showed data on a novel promoter — the DNA portion that initiates gene expression — for liver-directed gene therapies, indicating up to 40-fold greater protein expression compared to the referenced alternative. This promoter will be incorporated in AMT-180.

Source: **https://hemophilianewstoday.com/2018/11/26/gene-therapy-for-hemophilia-a-amt-180-may-treat-regardless-of-inhibitor-status-uniqure-says/**

Roche's Hemlibra shows sustained bleed control in children

BY ANNA SMITH

Roche's Hemlibra has shown sustained bleed control in a late-stage trial involving 85 children with haemophilia A.

Roche's Genentech has presented a primary analysis at the American Society of Hematology conference of a Phase III Haven 2 study evaluating Hemlibra (emicizumab-kxwh) prophylaxis in haemophilia A patients under 12 years of age with factor VIII inhibitors, including longer follow-up for once-weekly dosing and new data for less frequent dosing schedules.

Hemlibra was given priority review in the US back in June after Roche released interim results from the trial showing that after a median of twelve weeks of treatment, prophylactic treatment with the drug led to a clinically meaningful reduction in the number of bleeds over time.

Updated results now show that 76.9% of children receiving Hemlibra once weekly experienced no treated bleeds, while 23.1% experienced one to three treated bleeds. Furthermore, once-weekly dosing also reduced treated bleeds by 99% compared to prior bypassing agents in the prospective intra-patient comparison.

Roche said the new data also shows that 90% of patients who received Hemlibra every two weeks and 60% of those treated every four weeks experienced no treated bleeds, "demonstrating clinically meaningful bleed control at both dosing schedules."

"Children with inhibitors are at increased risk of life-threatening bleeds and may experience frequent, repeated bleeding into joints," said Guy Young, director of Hemostasis and Thrombosis Center, Children's Hospital Los Angeles, and Professor of Pediatrics, University of Southern California Keck School of Medicine, Los Angeles, California.

"These updated data from HAVEN 2 showed that the majority of children with haemophilia A with factor VIII inhibitors treated with emicizumab had zero treated bleeds across three different dosing schedules, reinforcing the ability of this medicine to provide sustained, effective bleed control."

Source: **http://www.pharmatimes.com/news/roches_hemlibra_shows_sustained_bleed_control_in_children_1271702**

The 2018 Australia & New Zealand social workers and counsellors group meeting

BY ROSS PATERSON

Thursday 11 October – Friday 12 October

Representatives attended this meeting from Victoria, Queensland, Australian Capital Territory, as well as two from New Zealand, Co-Chair Lynne Campbell, and Southern Outreach Worker Ross Paterson.

The first significant component of the meeting was a Professional Development workshop presented by Dr. Rob Gordon. Dr. Gordon is a Clinical Psychologist with 30 years' experience working with people affected by emergencies and disasters.

The theme of the workshop focused on the compounding nature and complexity of trauma, in which Dr. Gordon extensively explored the characteristics of cortisol and adrenalin stress, how they affect people, and, finally, identifying the priorities for First Aid workers when confronted with extreme levels of stress in traumatic situations. Dr. Gordon, an Australian, has travelled to New Zealand a number of times to provide advice to groups of people significantly impacted by the Christchurch and Kaikoura earthquakes.

The Australian members, with some contributions from absentee members, presented confidential reports, and Lynne Campbell presented a comprehensive report on behalf of the HFNZ community. All of the reports provided information on how the various participants undertake their work. Apart from the worker based in Canberra, and the New Zealand participants, all of the workers are employed by hospitals around the country. Trends and emerging issues, professional development, resource and information sharing, research and special projects completed, and camps and workshops were other topics traversed.

Resources discussed included a draft resource prepared by the Haemophilia Foundation of Australia entitled Telling Others about bleeding Disorders, which is a comprehensive information guide for women, girls, and their parents. Its focus considers issues of disclosure, the pros and cons of doing this, and discusses protecting your rights. It covered haemophilia, vWD and other severe bleeding disorders. Another resource recently developed was Female Factors, a new HFA resource for young women with bleeding disorders. Developed for the Australian environment the resource can easily be adapted to NZ. A number of our young women have viewed it and reported it to be both informative as well as providing a basic coverage of the full range of bleeding disorders. You can read more about the Female Factor earlier in this issue.

New Zealand shared the recent women's survey, findings, and poster, and presented on the recent Womens' Weekend held in Wellington.

Jane Portnoy a Social Worker based at the Alfred Hospital in Melbourne described her involvement in an Australian Twinning Project (as part of the GAP Programme) on Haemophilia in Vietnam. Jane described the work whilst discussing a series of photographs. The work was clearly very rewarding, but, not unexpectedly, the situation in a developing country such as Vietnam provided some challenges due to resourcing and language barriers.

Everyone in attendance endorsed the continuation of these meetings for the value they provided the workers undertaking these roles through networking, sharing of ideas and resources, as well as an opportunity to do this away from our day-to-day work.

AGM 2018

The Year Ahead...

April 12 - 15, 2019 (tbc)

- **National Family Camp**
  Venue to be confirmed

April 17, 2019

- **World Haemophilia Day**
  Activities to be confirmed

July, 2019

- **National Youth Camp**
  Venue to be confirmed

October 19 & 20, 2019

- **HFNZ AGM and Regional Office-holder Workshops**
  Roturua

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.