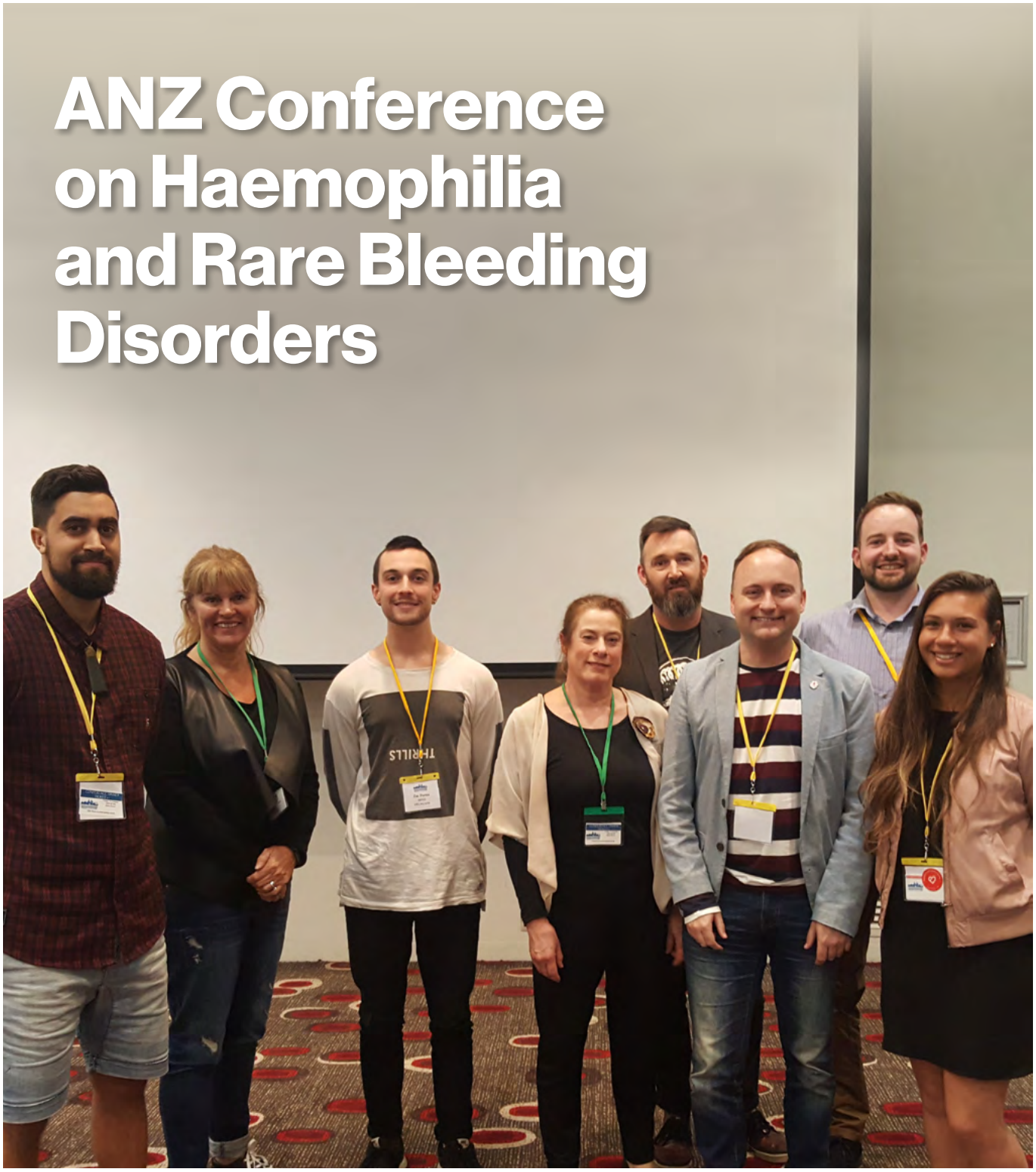
November 2017
Magazine of the Haemophilia Foundation of New Zealand. Volume 45 Number 3

Bloodline

ANZ Conference on Haemophilia and Rare Bleeding Disorders



Haemophilia Foundation of New Zealand Inc.
HFNZ

2017 HFNZ Adult Weekend «
Nepal Youth Twinning Visit «

Bloodline

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The H Word

Since the last issue of Bloodline the Foundation has been busy: we've had the HFNZ AGM, the ANZ Conference in Melbourne, and a Youth Twinning trip. That's not to mention all the usual things that happen for all our regions and groups at this time of year.

At the last AGM, the members and delegates unanimously approved the motions to seek additional finance over and above our existing property fund, so we can now search for a suitable property, confident that we can get the right property to suit our needs. The property search is underway in Wellington.

The ANZ Conference in Melbourne was a great opportunity for HFNZ delegates to get together with their Australian counterparts, learn more about one another, and hear about what's new in world bleeding disorder treatment and care. The conference had some excellent speakers, and covered a wide range of topics, from parenting, to gene therapy. As you'll read in this issue, our delegates all made the most of their time in Melbourne, and brought back all sorts of new information to share with you.

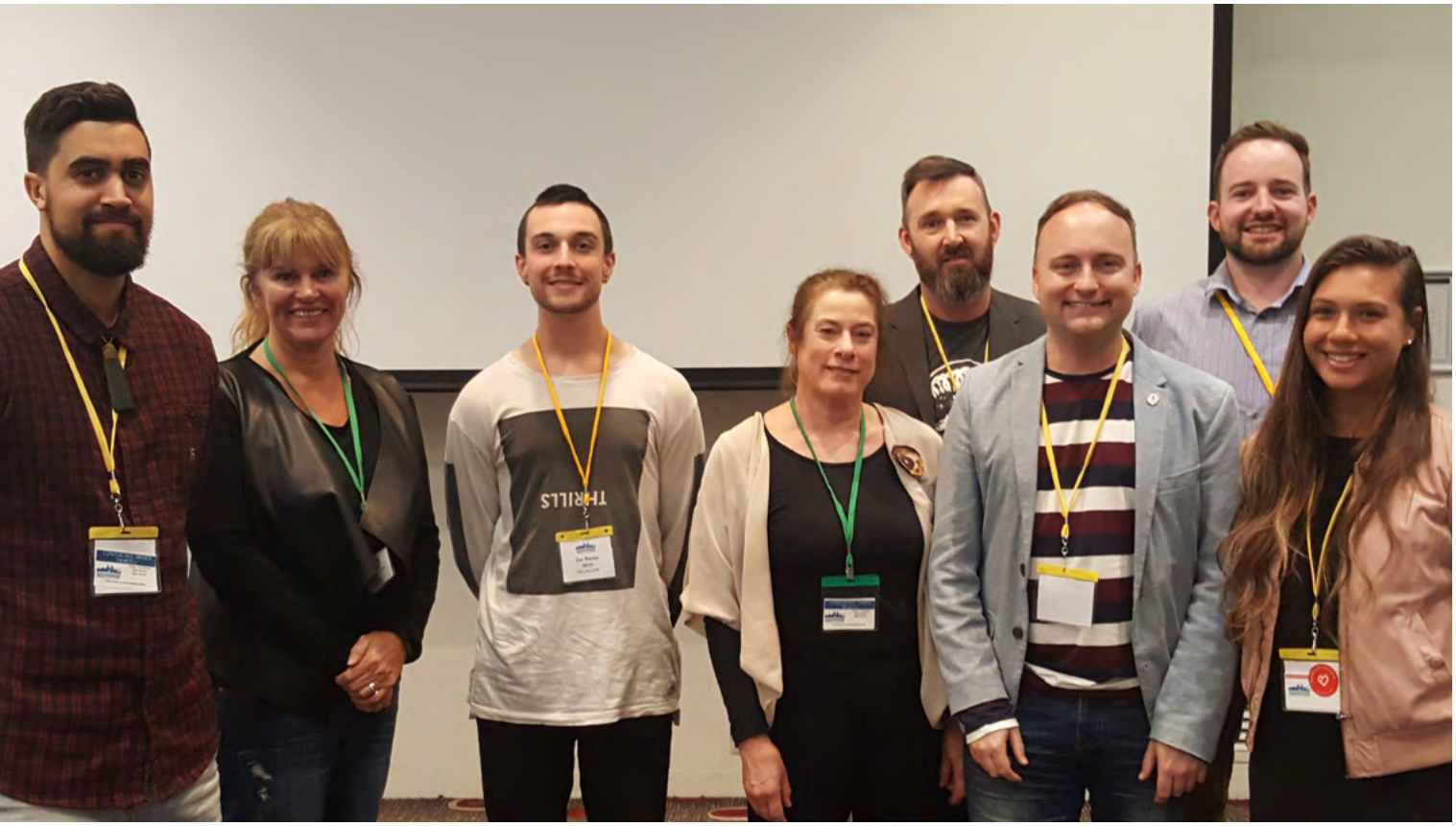
Another exciting piece of news came from the National Youth Committee. Right after the AGM, two of our youth members headed off to Nepal for an assessment visit as part of a prospective youth twinning, a new initiative of the World Federation of Hemophilia. They were to meet with local representatives of the Nepal Hemophilia Society, and with local health and government officials, before completing a report and formal application for twinning. I am pleased to say that their application was successful, and that there will be a formal 'twinning' with the Nepal Hemophilia Society. The twinning programme pairs up haemophilia patient organizations and treatment centres to learn from one another. The principle is that both organizations will grow and support one another over a defined period of time with

measureable development goals. This twinning will be an opportunity for more of our members to learn about care around the world, which also teaches us just as much about our own care, and how to improve it.

I would like to acknowledge the work of Josiane McGregor, who has recently moved on from her role as Southern Outreach Worker. Josiane was only with us for a year, but made a valuable contribution to the Foundation during that time. With Josiane's departure, we now welcome Ali Mitchell into the fold. Ali comes to us with experience in Nursing, research, and outreach services. Keep an eye out for her at upcoming events, and give her a warm HFNZ welcome.

This is the last Bloodline of the year, so it's an opportune time to wish everyone connected with HFNZ all the best for Christmas and the holiday season. As we get closer to the end of the year, many of our regions and groups will be holding events. I hope these all go well, and that as many of you as possible get the chance to touch base and unwind.

Deon York
HFNZ President



The HFNZ conference crew

ANZ Conference of Haemophilia and Related Bleeding Disorders

We had an enthusiastic team of six HFNZ representatives at the latest ANZ Conference on Haemophilia and Rare Bleeding Disorders in Melbourne earlier this month. Mahia Nightingale-Pene, Zac Porter, and Karl Archibald were our member reps; and Nicky Hollings, Lynne Campbell, and Phil Constable were the staff reps. Lynne also attended in her capacity as co-chair of the ANZHSWCG. HFNZ President Deon York also attended, as part of his WFH role.

The conference was well organised, and there were a number of great speakers, who shared some fascinating new information about bleeding disorders and other related issues.

21 Days to a Happier Family.

BY KARL ARCHIBALD

Former radio DJ turned psychologist and author, Dr Justin Coulson, presented the opening plenary session of the conference. Dr Coulson talked about ways to make sure your family is as happy and satisfied as they can be. He has six children, all girls, aged 3-18, so he considers himself eminently qualified, quite apart from his degree, to comment on parenting issues.

Regardless of where you reside in bleeding disorders, you reside in your family first. The question is; how do you make your family happier? Dr Coulson encouraged us to ask ourselves. "What is your vision for your family"? What do you want your family to be like? How should they relate to one another? Moreover, what does that actually mean for you?

Next, put that vision up next to your family's reality. How different is it?

Dr Coulson talked about his family and their morning reality, which is probably very much the same as the morning challenges faced by families all over the world: lost school bags, rushing about, finding shoes, gulping breakfast. What about that needs to change to make everyone happy?

There are four big feelings that underlie the decisions we make for our families: guilt, fear, anxiety, and anger. The biggest challenge is how to make good choices for your family; choices that help you achieve your vision, while not succumbing to those four key feelings.

The answer is to figure out what you want for your family, to have that vision, and then to make a plan for achieving it.

How are you coping at home? What are your daily challenges? How do you react when things go wrong? When things work well? Moreover, what can you actually do to be prepared for these times? Having a long-term plan, and sticking to it as well as you possibly can, is one of the keys to having a happier family.

The truth is; happiness is not having the children. Think about what having actually children does to your life. Researchers have found that we are most happy at the time they are born, then the challenges that follow, medical challenges, learning difficulties, bullying, just having a 16 year old, see us become less and less happy and satisfied. The same research shows that once the kids finally leave home our happiness levels increase again, back to pre-children levels.

However, while having children will probably reduce your personal happiness, you will also have periods of immeasurable joy, despite the challenges. There will be times with your family that you wouldn't trade for anything.

So, ask yourself; When has your family been the happiest?

In spite of all the crap we deal with in family life, it does feel good, even when it is hard. We get great joy from holidays, finding time for each other, relaxing, from fleeting moments snatched together, when they get home from school and are full of their great day. We love it when families come together, or when we are sharing a new moment with a young child. There is plenty to love and find joy in.

If you are going to make a plan to get to there, you need three central ingredients: Love, Limits, and Laughter.

1. Love

To a child, love is TIME. That being the case, then what does 'hurry up' mean? Does that tell them that they don't matter? Children are happier when they have time; your time, and the time to do the things they need to do. Research tells us that children who grow to be happy, contributing members of society always have someone who loves them unconditionally, who is there for them, who shows empathy, and gives them time. That person may be a parent, but it may also be a teacher, a relative, or an adult friend.

When you are most upset, pause and be in that moment with them, pause and look at the world through their eyes. Why are they upset? Stop and pay attention to what their needs are, if they are reasonable, then great, if they are not, then spend some time to discuss that. It's vitally important to talk with your children. The car is a great place to talk, and so is that period when they first go to bed.

Pro Tip: Parents that are sitting doing nothing are child magnets. Take the time, sit, and make the time for them. Just be "there", and the kids will come.

2. Limits.

If a child doesn't know how to read, swim, multiply, drive... we teach them.

If a child doesn't know how to behave what do we often do...? We punish them. How is that going to get the results we want?

It's important to get that relationship right. Don't punish them for not having a

treatment because they refuse to have their medication. Teach them why it's important that they do. Punishment makes children self-centred. They are not thinking about the lessons that you are trying to teach them, they are growing resentment; they are looking for a different way to do it next time, so they're not caught.

Look at the people that you trust the most. What kind of relationship do you have with them? You need to get the relationship right to foster trust.

So, what can help to get that relationship right with your children?

Time. As we've already heard, time = love.

Praise. This is a tough one. It's important to use praise very carefully, because it can undermine the very things we're trying to create. Too often we give hollow praise to the people that need genuine praise the most, it can then have a negative effect. Praising for the sake of praise can infer abilities that do not actually exist, raising expectations, and setting the child up for a fall later on. Be genuine in your praise, and make sure it's deserved.

Touch. Physical affection is vital to growing trust. In general, people associate touch with acceptance, closeness, and affection. So, hug your kids, rub their head, pat them on the back on your way past, and show them that you like them.

Explain. Give them a clear rational for decisions. Ask their opinion about how to make sure that unacceptable behaviours don't happen again. Being constantly told what to do is not fun for anyone, including your children. For kids, being involved in discussions about behaviour change makes it easier to own the change, and makes it more likely that it will happen.

Explore. What is the real reason they want it? "Because all my friends do it"? Do they really? Moreover, if they do, does that really make it a good thing to do? It's important to actually get to the bottom of things, and to allow your children to look past the immediate, and the short-term.

Empower. Sometimes you have to make a parent's call and just say no, like when your 14-year-old wants to go to a party where there will be drugs & alcohol. However, age and developmental stage is also an important consideration. It's a really good idea to talk through issues with your child, especially as they get older. Good conversations, where they can express an opinion, and hear what you have to say too, will often allow them to see the logical consequences of their choices. Conversely, such chats can also help you see things from their perspective, and help you to compromise; setting limits while empowering them to make good decisions.

Minimise control. Sometimes you feel like you just have to tell them what to do, "get in the car now, we need to go". However, having clear expectations, giving good information, finding out what they need to be ready in time, checking out any issues before they become a problem, these are ways that you can be collaborative with your kids, rather than dictatorial.

3. Laughter.

Just have fun. Many of us stopped being spontaneous at around the same time that we decided it was time to be responsible adults. There are some very simple ways to keep the fun in our family lives:

1. Music. Kids love music. Play music in the morning, in the car, when cleaning, have fun and dance, incorporate music into the time you spend with your kids.
2. Find the funny. Find the things that are fun and ways to have a laugh. This also includes Dad jokes. Lots of Dad jokes…
3. Stop, Drop, and Roll. Get home, get on the floor with them, and just play. This will change the way your kids thinks about you, and change the family dynamic
4. Establish traditions. Find something that you can do regularly, like super-Sunday outings, screen-free fun time, camping, tramping, anything you can do regularly that makes for a fun family time. Make a tradition, make it predictable, so your kids can look forward to it, and just have fun. Spend time, show love, set limits, and have fun.

What sorts of things can your family do for fun?
Remember:

- We'll never have time for everyone all the time.
- Stop wanting to please everyone, and just be normal
- Stop wishing for the impossible, like skipping treatment and never giving another needle
- Get past feeling guilty all the time.

People often expect family life to be a picture postcard, one big gold nugget. However, quite often it's hard work, tiring, challenging, and aggravating. Nevertheless, if we take the time, and make the effort, we find that family life is built of lots of gold flakes, memories that combine to make that ideal picture post card, that massive golden nugget.

Now it's time for you to start to make a plan.

HCV and HIV

BY PHIL CONSTABLE

This session looked at the current state of HCV and HIV treatment. The session was divided into four sections.

First, we heard a personal story from a man named Simon. Simon is an architect with haemophilia, who contracted HCV from a treatment product. He is constantly surprised that people are still choosing not to take up the new direct acting antiviral (DAA) treatments like Harvoni. However, he understands that previous experiences of treatment with interferon may have made people suspicious of treatment in general. In fact, Simon expressed his anger at just how ineffective past treatments have been. He noted that the low cure-rate, and the sometimes-severe side effects, could lead to people being unwilling to take up treatment in the future. He did treat with interferon, and cleared initially, but his HCV returned. As a result, he lost a year of his studies, a year he'll never get back.

On the other hand, for him, the DAA treatment was like magic. He had no side effects whatsoever, and no down time due to treatment. Like many sufferers, Simon had tried to ignore his symptoms, and to avoid any bad news regarding his HCV by missing appointments and not communicating with his doctors. The DAA treatment evaporated the shame and worry he'd felt for so many years.

Following Simon's story, we heard from Associate Professor Joe Sasaduesz from the Peter Doherty Institute for Infection and Immunity. He asserted that DAAs are close to the perfect HCV drug, and that HCV is the first viral disease that we have manage to cure. Prior treatments had significant side effects, required prolonged courses of therapy, and were not particularly effective. DAAs, however, have minimal to no side effects, are around 95% effective, and usually require just a 12-week course of treatment. They are also just as effective in cases where there is an HIV co-infection, although care needs to be taken about drug-drug interactions. There is still an issue with genotype 3 HCV, though, which often requires a longer course of treatment, and isn't as effectively treated.

For people who are HIV positive, if they are taking ART, and have an undetectable viral load, they effectively have no risk of sexually transmitting the virus to an HIV negative partner. This is a huge step forward from the, relatively recent, days when contracting HIV was effectively a death sentence.

The newest DAA currently available is Epclusa, which is between 98 and 100% effective across all genotypes. Again, the side effects are mild to moderate, and they only affect around two thirds of patients.

In Australia these drugs can be prescribed by GPs, there is no restriction on liver disease or drug and alcohol use, no cap on the number of patients, and retreatment is available for reinfection. Something for NZ to consider.

Next at the podium was Associate Professor Edwina Wright, infectious diseases physician and clinical researcher at the Alfred Hospital and the Burnet Institute. She spoke about the advances in HIV treatment and care.

Prof. Wright suggested that, worldwide, new HIV infections are predicted to end by 2030. In Australia, they're looking at 2020. The strategies to get there include regular testing, drop-in centres, PrEP – pre-exposure prophylaxis, PEP – post-exposure prophylaxis, disclosure, treatment-as-prevention, and IVF.

The big thing these days is treatment-as-prevention. What this means is making sure that people are treated for their HIV, using antiretroviral agents (ART), as soon as it is detected. The treatment is primarily for their own health, it will reduce viral load to undetectable levels, and a positive side effect is significantly reduced transference.

For people who are HIV positive, if they are taking ART, and have an undetectable viral load, they effectively have no risk of sexually transmitting the virus to an HIV negative partner. This is a huge step forward from the, relatively recent, days when contracting HIV was effectively a death sentence.

Pre and post exposure prophylaxis involves HIV negative people taking ART before or directly after sexual intimacy, intravenous drug use, or conception. PrEP's efficacy rests upon adherence. That means you must stick to the prescribed regime. Daily dosing is associated with a 99% reduction in HIV transmission.

The message from Prof. Wright was that these days it is possible to effectively eliminate the risk associated with previously risky behaviours. Treatment-as-prevention with antiretroviral drugs, pre or post exposure, whether you are HIV positive or negative, virtually eliminates the possibility of contracting or transferring the virus. She told us that treatment-as-prevention is even more effective than condoms. Unfortunately, despite World Health Recommendations, PrEP is not available, or even being studied, in New Zealand.

The session wound up with a personal story from Anthony, who has severe haemophilia, is HIV positive, and has been treated for, and successfully cleared, HCV.

Anthony talked about how he acquired HCV & HIV via tainted blood products. He missed a lot of school as a kid due to bleeds, and suffered ostracism and bullying due to his Haemophilia. He chose to disclose his HIV to friends and family when he was in year 12. This was the beginning of an ultimately very rewarding process, which culminated in his public disclosure via social media on World Aids Day 2016.

Anthony was very proud of where he had got to in his life. He has a wife and children, and he has his HIV under control. His life has been hard at times, but he is moving in the right direction.

Over all, this session painted a picture of hope and of success. HCV is now nearly 100% curable, and HIV is controllable to the point where we can expect a zero new infection rate in our lifetime.

Genetic testing

BY LYNNE CAMPBELL

This session was in two parts:

Family Planning and the role of the Genetic Counsellor

Carolyn Cameron, genetic counsellor at Monash Genetics, provided an introductory overview of diagnosis, genetic testing, counselling support, and informed decision-making, using haemophilia as an example.

She contrasted the X-linked inheritance of haemophilia with the vWD dominant inheritance pattern, and also noted the impact of an autosomal recessive Rr x Rr inheritance pattern, such as in FVII deficiency, where both parents carry the gene (although the parents are not affected there is a 25% chance a child may get both recessive genes rr).

The role of the genetic counsellor is to background the situation, assess risk, clarify understanding and expectations, and oversea a management plan for the pregnancy. Ideally and where possible, initial testing is done on the proband - the first affected individual. Approximately 98% of mutations are detectable.

Informed decision-making is crucial. In FVIII and FIX clotting deficiencies factor levels are not accurate enough to predict Carrier status.

Prenatal diagnosis can be determined through chronic villus sampling, where placental tissue is analysed, or via amniocentesis, which is performed a little later in the pregnancy and the results take longer to come back.

A mother's decision to terminate is usually based on tests such as FISH (Fluorescence In Situ Hybridization) for chromosomal abnormalities. The result may determine whether the patient will terminate the pregnancy; there is no right or wrong decision in the eyes of the genetic counsellor.

Non-invasive prenatal testing is a new test now available to predict if the baby has a disorder. A simple blood test of the mother shows up the DNA of the foetus. This test also indicates whether the foetus is male or female. This can help the patient decide whether to have other testing, such as the FISH test. It is less traumatic as it can be done earlier in pregnancy than an amniocentesis.

Pre-implantation genetic diagnosis (PGD) utilises IVF technology. The mutation must be identified beforehand, and the diagnosis of an embryo at the 16-18 cell stage is highly accurate in determining whether a mutation is present. It also identifies the sex of the embryo.

Informed decision-making can result in a conflict of emotions, beliefs, and values between individuals and other family members. Clinical severity tends to run true in families, and consent to investigate a mutation includes permission to use other family members.

The Genetic Counsellor develops a pregnancy management plan to suit whatever decision is made.

The pathway to genetic testing- Dr Matt Hunter-Clinical Geneticist

This presentation focussed on:

- **The genetic causes of bleeding disorders**
  - In females with two XX chromosomes, randomly only one X of the two is activated or silent. A skewed x-inactivation can cause bleeding in women.
  - the most common mutation in Haemophilia A is caused through Intron 22 inversion (approx. 45%). By contrast in Haemophilia B 50% of mutations are new, however Inhibitors are rare
  - vWD is the most common, most people are autosomal dominant unless in the case of vWD type 3 which requires both parents to have the vWD gene.
  -platelet disorders and clotting factor disorders are many and varied.
- **Advances in genetic testing**
  -Next generation sequencing is rapid, cheap, and rapidly becoming the test of choice for many labs. It is based on a simple prenatal test of the mother's blood, as DNA from the baby is detectable in the blood of the pregnant mother
  -a single test is not too far away
- **The value of Genetic testing**
  -to clarify diagnosis, detect carrier status, and predict inhibitor formation. Medics can be strategic about the treatment of likely Inhibitors and allergic reactions to blood products
  -utilise gene therapies and genome editing in the future
- **Genomics**
  -genomic tests will detect inversions, single nucleotide changes, deletions and duplications, as well as modifiers of severity, indicating immunity to factor replacement and response to medications
- **Gene therapy**
  -only a small increase in Factor provides a big effect on clotting. In time, it will be possible to insert extra copies of the required gene into the genome to increase clotting

- **Genome editing**
  -this is another new technique, which cuts the DNA to remove the faulty gene sequence and replaces it with the correct sequence.

Ethically there are issues to consider, and spontaneous mutations are happening in the body all the time.

Youth Myth Busting

BY NICKY HOLLINGS

An engaging, open, panel discussion around sex, employment, and tattoos.

A panel of people with bleeding disorders, parents, and health professionals discussing whether tattoos are safe for people with bleeding disorders? Overall, the feedback around tattoos was that they are no more dangerous for someone with a bleeding disorder than without, what makes tattoos dangerous for the wider community is dangerous for all.

We heard feedback that it can be very difficult at times for youth to have a discussion with their HTC team due to the lack of privacy. It's important for the team to understand that a quiet place to discuss personal issues will help with a range of challenges, including trust and understanding, which contribute to treatment adherence. Don't have these conversations in the openness of a hospital setting.

Members of the panel described the impact of disclosing a bleeding disorder in the workplace as being overall a good experience, where managers have been supportive. Unfortunately, there had been situations where managers had disclosed to the wider workplace, which had led to feelings of discomfort and hurt. There had been experiences of people not getting employment due to not being able to get workplace insurance (this is an Australian situation), and people losing work due to bleeds. When discussing this, youth talked about the importance of being your own advocate, or bringing in a professional who can advocate for you.

The overall theme of the session was to let youth live their lives, and to live with the responsibilities of their choices. At times this means understanding that some youth will not be responsible for taking their treatment.

Von Willebrand disorder – Diagnosis, treatment, and care

BY PHIL CONSTABLE

The key speaker for this session was Prof. Paula James, from the departments of Haematology and of Pathology & Molecular Medicine at Queens University in Ontario Canada. She looked at some history, some approaches to diagnosis, the types, and sub-types of vWD, and management.

Von Willebrands Disease (vWD) was first described in 1926, and is the most common bleeding disorder. It affects as many as one in 1000 people, although many may have no idea that they're affected. It is characterised by excessive bleeding of the skin and mucous membranes. Von Willebrands is caused by the lack, or dysfunction, of von Willebrands factor (vWF), which sticks platelets down to sites of vascular injury. Von Willebrands factor also carries and protects Factor VIII, so a secondary characteristic of vWD can be low FVIII levels.

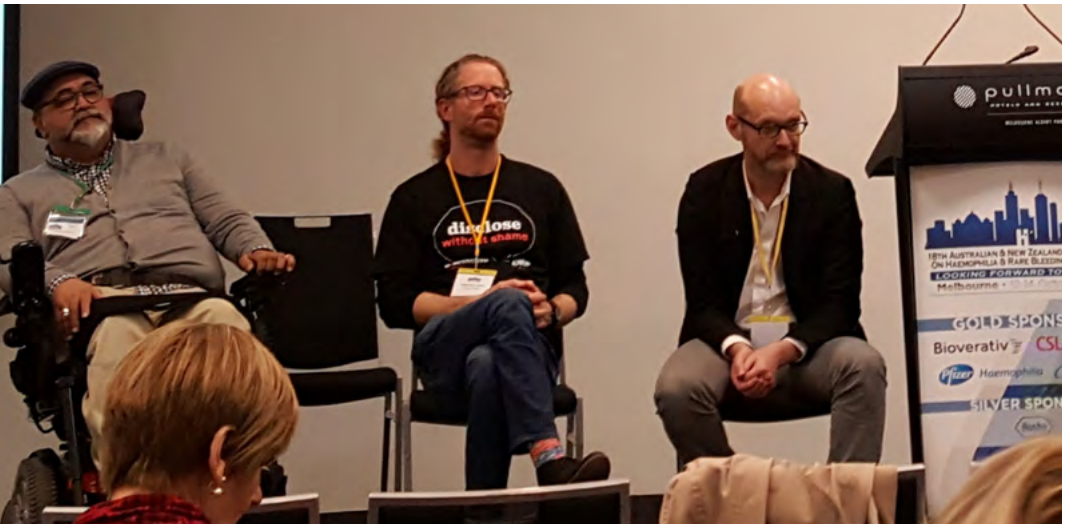
There are a number of symptoms that can indicate vWD. These include heavy periods and post-partum bleeding in women, gastro-intestinal bleeding, frequent or severe nosebleeds, excessive bleeding from cuts, and, in severe cases, joint and muscle bleeding. Assessment is usually based on a comparison with what is accepted to be normal. However, women bleed physiologically every month, and every woman is different, so it can be hard to determine whether bleeding is excessive.

One clear indicator is family history. However, not all type 1s are positive, and not everyone with vWD parents gets it themselves. In addition, there can be different expressivity between family members. One may get occasional bleeding noses, while another may have a three week period every month.

The definitive way to diagnose is via lab test. Unfortunately, the tests are not widely available, are very difficult to administer accurately and consistently, and can be influenced by a range of other factors. Stress, medications, exercise, pregnancy, and age can all elevate vWF levels. So, if someone is injured, and bleeding, they may test normal due to stress, when in fact they usually have very low levels.

Von Willebrands is generally classified into three main types. Type 1 is the most common, affecting 70-80% of patients, and is characterised as a person not having enough vWF. Type 2 has various sub-types, but is broadly categorised as having vWF that doesn't work properly. Type 2 affects 15-30% of patients. Type 3 is very rare indeed, and is where the patient has no vWF at all.

So, why is it important to understand the characteristics of vWD? The main reason is to improve quality of life. The fact is that in most cases the symptoms are very treatable, and good management can mean that patients have a relatively normal life.



It is also important to know what to expect when undergoing surgical procedures, including dental work. In addition, there can be harm from over diagnosis.

The key to vWD management is to get an accurate diagnosis, then to create an individualised treatment plan.

In women, in terms of heavy menstrual bleeding, the treatments are either medical or gynaecological. The medical options include using the contraceptive pill, patch, or ring to regulate bleeding, or using tranexamic acid at period time. Interestingly, Prof. James suggested that reducing the number of periods a woman has per year is a safe and effective way to mitigate heavy bleeding during menstruation. There is no need to have a period every month, when you can control it using the pill. Many women work up to only menstruating every three months. Prof. James stressed the need to build up to this, under medical supervision.

Gynecologically, women can use a hormonally coated intra uterine device (IUD), known as a Mirena, they can undergo endometrial ablation, where the lining of the uterous is either burnt or frozen off, or they may choose to have a hysterectomy.

The treatment Prof. James most often prescribes is Desmopressin (DDAVP). This medication causes a release of stored vWF, and may be administered as an injection or a nasal spray. This works for most patients, although there is a poor response for severe type 1s, type 2Bs, and all type 3s. This treatment can only be used intermittently, because the body needs a chance to build up vWF stores again. There are also warnings about fluid intake. Desmopressin causes fluid retention, so drinking normally in the first 24hrs after dosing can lead to reduced sodium levels in the blood, and seizures.

Many people use vWF concentrates. These are currently plasma derived, and are a combination of vWF and FVIII. Apparently, there are recombinant concentrates on the way, which will be just vWF. These can be combined with FVIII when required. Using vWF concentrate as prophylaxis will help to control bleeding, improve joint health, and limit recurrent mucocutaneous bleeds.

One particularly difficult symptom to treat in angiodysplasia. This is characterised by intractable gastrointestinal bleeding, bleeding from the mucous membranes lining the bowel and gut walls. Von Willebrands patients are at increased risk of this type of bleeding. Angiodysplasia does not always respond well to vWF concentrates, meaning increased dosages, and treatment that is more frequent. However, there are some novel agents being successfully used.

For women with vWD who are planning to become pregnant, it's important to correct any iron deficiency first, and to get yourself some good prenatal supplements. Remember, a healthy mum leads to a healthy baby. Before pregnancy, adjust your medications, stop your birth control and consider tranexamic acid. Meet with clinicians involved in managing your bleeding disorder, and consult with childbirth experts. It's important to have a plan around delivery, and to understand what type of vWD your baby is at risk of inheriting.

Following Prof. James, a young man named Brendan shared his personal story.

Brendan is 28, and, we were later assured, is pretty understated about the actual severity of his vWD. He talked about choosing not to restrict his activities, and relying on treatment to fix him when he needs it. He had an active childhood, and continues to enjoy football, motorbikes etc. Brendan has travelled and worked overseas, and, although arranging travel insurance was always a trial, he has never had any significant issues when away.

Images top to left & right:
A panel discussion at ANZ Conference

Brendan reiterated Prof. James' point that vWF levels rise at times of stress or injury, and that his vWD has become less severe as he's got older. In general, he reckons he's not too badly affected.

The session wound up with Dr Mandy Davis talking about the local HTC and some of the work done at the Alfred Hospital in Melbourne.

Evolving concepts in pain management

BY LYNNE CAMPBELL

This session was in three parts:

Understanding pain – Martina Egan-Moog

Pain is a complex experience. It is based on our perceived balance of safety vs danger and the need to protect our bodies. We are "bioplastic" in as much as pain is changeable. Danger signals can start from almost any tissue in the body. Martina Egan-Moog said our bodies have danger sensors, NOT pain sensors and the brain determines what we detect.

In coping with the many signals coming into the brain, the brain identifies evidence of danger vs evidence of safety.

She also discussed DIM vs SIM

DIM = danger in me (this is partially cultural). Anything dangerous to body tissues, life lifestyle, job, happiness, and functionality.

SIM = safety in Me. Anything that makes you stronger, better, healthier, more confident, more sure and certain within and about yourself.

Pain thresholds lower if the person is in the SIM zone. Pain is constructed as a protective experience and is changeable. Over time, the nervous system becomes more sensitive and more protective.

The term chronic pain is gradually being replaced with "persistent "pain as the more preferable term.

This speaker's advice was to make bioplasticity work for you by:

- identifying DIMS and SIMS
- Modify and remove DIMS
- Strengthen and gather SIMS

Evolving Concepts in pain management – Associate Professor Carolyn Arnold

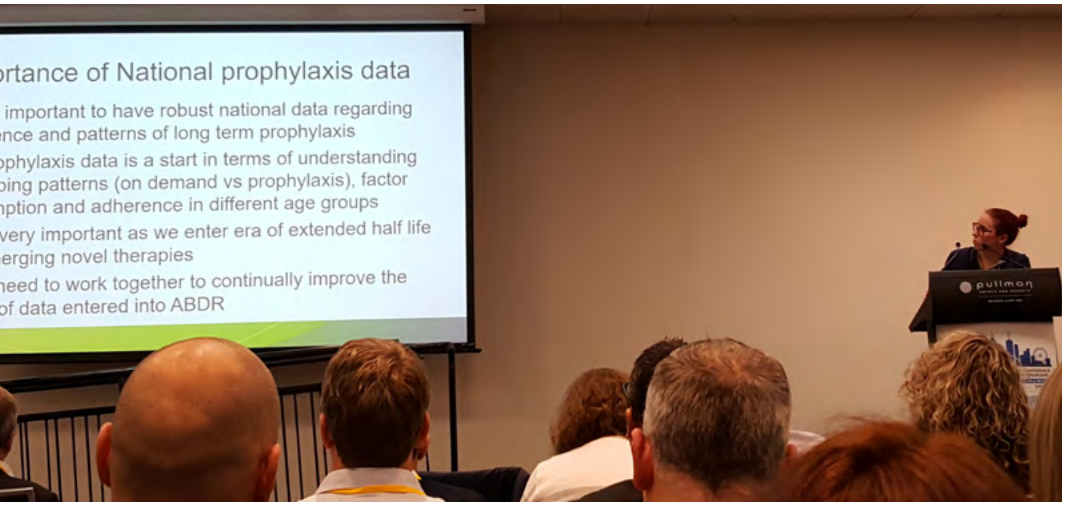
The rehabilitation of pain requires a whole person approach to managing pain over a lifetime. Medication is only a small part of how we manage pain.

This speaker talked about the history of the treatment of pain over 20+ years and the move towards a more wholistic approach to the management of all pain. She described acute pain vs persistent/chronic pain in joints and muscles and the use of analgesics and PRICE for treatment of acute pain, as well as the need for rehabilitation after rest. She urged caution in the use of strong analgesics, explaining that they work only in the short term.

Prof. Arnold also discussed the fact that pain is not necessarily always caused by bleeding. Nociceptive pain is persistent pain from degenerative joint disease, while neuropathic pain is from nerve dysfunction as the result of bleeds. An acute bleed can merge into chronic pain.

Her main message was that individualized treatment is best; opiates should be used only rarely as they do not control chronic pain long term and have many negative impacts with long-term use.

Her recommendation is that an individualized wholistic approach to management of pain should be encouraged, which includes exercise as a balanced activity, weight control, mood optimization,



and patient education. Medication is only part of pain management.

Clinical application of modern pain sciences for PWBD, an "active approach" – Cat Pollard - Physio

One in five people in the general population experience chronic pain. This is much higher in Haemophilia. Acute pain is short lived and can be fixed, while chronic pain is long lasting and not so easily fixed.

There is confusion in the bleeding disorders community because from an early age people with haemophilia have been encouraged to treat; however, not all pain is the result of bleeding.

Cat described the "biopsychosocial model of health" where Bio focuses on the periphery i.e. surgery, orthotics, physio, exercise, diet, and lifestyle etc., Psycho focuses on relaxation, mindfulness, acupuncture, exercise, diet, and lifestyle etc. and Social which focuses on education of the patient, impact of the bleeding disorder, work, family, school, support networks etc.

Her presentation focused on the importance of lifestyle and balance in parallel with the more traditional, and not so traditional, treatment options available to people with bleeding disorders. Psychological management strategies such as relaxation and mindfulness help to wind down the nervous system, reduce stress, and improve coping skills. Tissue is only one part of the picture.

There is strong evidence that in terms of pain relief, exercise is as effective as, and often more effective than, medication for pain relief. The fear of pain can be more disabling than the pain itself.

Exercise is extremely important for joint function. The production of endorphins during exercise also reduces stress and anxiety and improves sleep.

The aim is to avoid a flare-up through graduated exercise.

Diet and lifestyle are inexorably linked where increased body weight leads to increased joint loading and risk of bleeding, which leads to damage and increased pain, which leads to reduced ability to be physically active, which results in increased body weight.

Realistic goal setting is the key. It is important to exercise with a goal in mind for added motivation. Sometimes pain is not the focus when a person is busy doing something else.

Prophylaxis

BY ZAC PORTER

Dr Jane Mason: Current real-world prophylaxis practice

Dr Mason discussed a Swedish longitudinal study that compared on-demand treatment vs prophylaxis for treating bleeds. The study found 50% of the on-demand group has existing joint damage.

She also discussed an Australian randomized study that compared prophylaxis vs on-demand treatment for Haemophilia. The study found that on demand patients bled significantly more than prophylaxis patients did, mean annual joint bleeding rate was much higher for on demand, and prophylaxis reduces joint damage in children.

Bottom line/take away message: Prophylaxis reduces bleeds significantly. It reduces bleeds but does not eliminate bleeds.

Issues with prophylaxis:

- What's the best age to start? Aim for 3 years old
- What about dosage and dose intervals?
- Inhibitor risk from early prophylaxis?
- Should you use prophylaxis for moderate haemophilia?

- What about prophylaxis for aging individuals? Limited research in this area.
- It's tough to achieve zero bleeds.
- What "allowable" sports/activities are there?

There is a significant drop off in joint bleeds if you can get factor levels above 1%, and further drops at 3-5% and 10%. Researchers are unsure why just yet.

It's important to remember that prophylaxis doesn't prevent regular lifetime joint damage. For people with bleeding disorders, joint damage normally starts in the ankles with ankle atrophy. This raises the question, should there be aggressive early ankle targeting?

Prophylaxis regimes are most effective when tailored to individual's needs, including age, activity levels, etc. Half-life studies are often used to determine factor levels. However, factor levels are not the only marker of bleeding severity, and other factors should be considered. This leads to higher overall cost of product but produces economic savings in other areas.

Sumit Parikh: National prophylaxis data analysis (Australia-ABDR)

Australia's National Blood Authority developed and funded the Australian Bleeding Disorder Registry (ABDR) in December of 2008. In 2012, ABDR introduced a patient recording module; previously no patient reported data about prophylaxis was available in Australia.

ABDR aims to investigate prophylaxis practices in Haemophilia A + B. Data includes weight, age, baseline factor levels, etc.

There are currently 718 haemophilia A patients using ABDR, 76% severe and 23% moderate. Of those, 82% are on prophylaxis, and 18% are on demand. For haemophilia B there are 166 patients using ABDR.

According to the data, the majority of haemophilia A patients is on prophylaxis 3 times per week, whereas, the majority of Haemophilia B patients is on prophylaxis 2 times per week.

There is still work to do with adherence to prophylaxis schedules. Currently in Australia, 23% of patients use only 75% of the prophylaxis treatment they're prescribed. A big contributor to this number is a significant drop off in prophylaxis adherence in adolescence.

Following Sumit's presentation a young man named James Rogers shared his personal experience via a very brief video. He is a great example of attitudes to adherence anong adolescents. James has severe haemophilia A. He is meant to take his treatment as prophylaxis every second day; instead, he only treats once per week. James claims he would rather do homework than have factor.

Dr Huyen Tran: Medical matters for people as they age

Dr Tran is a Haematologist at Alfred Hospital Australia.

He spoke first of a 97-year-old WW2 pilot with severe haemophilia A. Previously, getting to this age was unheard of. Now we have an aging population, and regularly see patients between 90-100+ years old. Haemophilia patients now have normal life expectancies. In the Netherlands, over 40% of haemophilia patients are over 50 years old.

Dr Tran emphasised that life expectancy with haemophilia is enhanced by prophylaxis and by the being fewer limitations to surgery now.

Going forward, what can be improved in the next 10 years?

Dr Trans says that it is important that we're teaching the next generation about bleeds, and about bleeding disorder management. He would also like to see an improvement in the child to adult health system transition.

The fact is; if you have haemophilia, your quality of life is generally not as good. As age increases, there are more haemophilia related issues such as arthropathy and HIV, as well as increased non-haemophilia related issues like cancer.

Haemophilia related issues can include pain management, arthritis, osteoporosis, arthropathy, orthopaedic surgery due to degeneration, rehab and physical therapy. Happily, HCV is now virtually gone, and HIV is very manageable.

The latest novel therapy for haemophilia A with inhibitors is Emicizumab. This medication is prescribed as prophylaxis, via a sub-cutaneous injection once per week. To date, 63% of those on trials report no bleeds, and 87% report a reduction in the frequency of bleeding episodes.

Unfortunately, just like the rest of us, people will still experience non-haemophilia ageing issues. Obesity is increasing worldwide, and may be the next health crisis that needs confronting. Likewise, heart disease is an ongoing concern

Cancer rates continue to be high, and aging haemophilia patients are particularly susceptible to cancer. Your GP is the most important point of contact for screening, not your haematologist.

As you age, there is also increased risk of venous thrombosis, and the associated risk of haemorrhage, which is of particular concern to people with bleeding disorders.

In general, significant improvements in care leads to an ageing bleeding disorder population who become more at risk of non-bleeding disorder issues. Haemophilia centres remain central and critical to the treatment and care of people with bleeding disorders.

Let's Talk Period: Women and Bleeding

BY MAHIA NIGHTINGALE-PENE

The presentation from Professor Paula James was vey informative. She spoke about women and bleeding disorders, her effort to raise awareness about women and bleeding disorders, and her project 'Let's Talk Period".

Studies show that as many as 1 in 1000 people may have an inherited bleeding disorder. Many of those remain undiagnosed. Women with bleeding disorders are at particular risk of remaining undiagnosed due to a general lack of understanding around what constitutes normal bleeding, particlularly menstrual bleeding. In addition, there is often social stigma around open discussion of menstrual issues. This can lead to significant delays between the onset of abnormal bleeding, and recieving appropriate medical attention.

Prof. James looked at some of the challenges facing women with bleeding disorders, and ways the she and her team approach assessment and management of these women. She also emphasised the distinction between the symptoms suffered by men and women, and how imporetant this is to the bleeding disorder community worldwide. Women physiologically cannot be 'bleed free' and approached to care must recognise this.

Menorrhagia

Prof. James spoke about Menorrhagia. Menorrhagia is the medical term for menstrual periods with abnormally heavy or prolonged bleeding. Menorrhagia can cause a negative impact on the quality of life; with those who have it having to be absent from work and school, it causes 2/3 hysterectomy in women of reproductive age and it causes iron deficiency.

Why should you get diagnosed? Because symptoms are treatable and you deserve to have a positive quality of life.

Let's Talk Period

Women with bleeding disorders are particularly at risk of going undiagnosed, as there is a general lack of understanding about the difference between normal and abnormal bleeding, particularly menstrual bleeding.

"Let's Talk Period" is Dr James' project that is an easy access website. It is educational and also has a handy tool that helps women understand whether current, or previous bleeding episodes are normal or abnormal. The handy tool is called the Self-BAT (self-administered bleeding assessment tool) and it takes no longer than 4 minutes to complete.

So why should you visit the website and take the test? Because you may learn something new and the test could benefit your understanding of your menstrual bleeding.

Healthy Joints in Adolescence for Life

BY PHIL CONSTABLE

The key speakers in this session were Alison Morris, senior musculoskeletal physiotherapist at the Princess Hospital for Children, and Abi Polus, senior clinical physiotherapist at the Alfred Hospital.

The title of this session was 'Pain is not always a bleed: Recognising other causes of pain across the lifespan", which gave a clear indication of where the session was going.

Alison Morris led off by addressing the fact that children and adolescents are not mini adults, and that many of the injuries that occur are primarily due to physiological differences in the growing skeleton. There are growth plates that are responsible for longitudinal bone growth, and injuries can induce growth disturbance in the growth plate resulting in deformity.

We saw that, in children, many of the joints in the ankle knee, and shoulder are relatively unformed. The growth plates are often at risk of avulsion, where the plate slips off the end of the bone due to trauma. In ankles, there is a real deficit in hard bone, as feet grow, and limbs develop. These connections can be very fragile, and joint pain is often related to damage here, rather than due to bleeds.

Alison then proceeded to run us through a selection of common injuries in young people that are related to immature bones and connective tissue, the presence of which may be mistaken for a bleed. In fact some of these injuries may lead to bleeding into the joint, however, bleeding will not be the primary cause of pain.

The first injury is a slipped upper femoral epiphysis (SUFE). Here, the ball of the femur slips off the top of the bone. This injury is historically seen in overweight boys between 9 & 15yrs, with a peak at 12 – 13 yrs. It's usually a progressive injury, but may occur suddenly. Treatment for a SUFE injury is pinning the ball back onto the top of the bone.

The next injuries are avulsion fractures of the pelvis, where a section of the bone is pulled off the pelvis where the muscle attaches to it. This occurs because, in young people, the tendon is often stronger than the young bone. Again, this is more common in boys, at a mean age of 14.5yrs.

Another group of related injuries are known as traction apophysitis. An apophysis is a protuberance on the bone where a muscle tendon is attached. These traction injuries are caused by repetitive changes in load. While the term 'itis' usually refers to inflammation, MRIs confirm that these injuries are primarily non-inflammatory.

The most common of these injuries are Osgood Schlatters and Sinding-Larsen Johansson in the knee, and Severs in the ankle. However, a traction injury can occur at any tendon attachment to apophysis.

Alison also noted that the number of reported anterior cruciate ligament (ACL) injuries in immature athletes is increasing in Australia. This could be down to increased participation in sport, greater awareness of the nature of the injury, and improved imaging techniques. This injury usually comes from pivoting on a slightly flexed knee, or from hyperextension. In young people, an ACL injury can lead to growth plate arrest.

Next up was Abi Polus. She began by noting that ACL injuries are far more common in adults, and can include a bleed into the knee, even in patients without a bleeding disorder. Eighty percent of ACL tears include associated bone bruising, which can also have implications for people with bleeding disorders.

Abi went on to talk about the normal effects of ageing on the musculoskeletal system. Believe it or not, this starts going downhill from about age 30! Bone density starts to diminish in men and women, the cartilage inside joints becomes thinner, connective tissue becomes more rigid and brittle, and there is a gradual loss of muscle mass and muscle strength.

Abi has a particular interest in shoulders. She noted that the shoulder is an incredibly complex joint that needs a number of things to work in concert in order for it to function correctly, and without pain. She likened the joint to a seal balancing a ball on its nose, because the ball of the humerus bone is not enclosed in a full socket. The point was that joints are complex mechanisms, and that there can be a number of factors, other than a bleed, that can lead to pain.

Tendinopathy is a common source of joint pain, and becomes more common from the age of 30. Clinical presentation includes tenderness on palpation, and pain, often when exercising or with movement. For example, anterior knee pain can be caused by patella tendinopathy. Knee pain can also come from poor muscle conditioning, which pulls the patella out of alignment and stops it running smoothly in its groove.

Osteoarthritis is another common source of joint pain. While it may be the result of bleeding onto the joint, it may also be due to genetics, weight, or age. The best way to manage osteoarthritis is through exercise and physiotherapy, and by weight management. In severely debilitating cases, medication and/or operative intervention are also considered.

Next, the speakers moved on to looking at ways to make decisions around sports participation for people with bleeding disorders.

It is widely accepted that participation in athletic activities is very important in maintaining a healthy lifestyle, and in preventing adverse health outcomes like cardiovascular disease, obesity, type 2 diabetes, osteoporosis, and mental health conditions. Various guidelines exist to assist in decision making around sports participation for people with bleeding disorders. These are often based on perceived risk:

- The probability of contact or collision
- The frequency of recorded injury
- The incidence of injury
- The potential for catastrophic injury

The question is; are we being too simplistic?

In sports medicine, decisions are constantly being made around when an athlete is ready to return to play following significant injury. These decisions are based on best research evidence, clinical expertise, and the athlete's values and circumstances.

One assessment tool in common usage is the Strategic Assessment of Risk and Risk Tolerance (StARRT) framework. This tool assesses current tissue health against the activity the patient participates in, alongside any factors that modify either the health risk or the activity risk. StARRT can be used to assess activities suitable for people with bleeding disorders, where the goal, rather than return to play, is participation.

Step 1 is to assess health risk by looking at:

- Factor Level
- Presence of Inhibitors
- Bleeding History
- Joint Status

- Prophylaxis Regime
- Level of Physical Fitness
- Additional Medical Issues
- Geographical Location From HTC
- Level of Compliance with Existing Medical Regime

Next, step 2 is to assess the level of risk the desired activity presents. This is done in conjunction with the patient, the HTC team, the sports coach, and the parents. They will look at:

- Type of Sport
- Position played, because different positions may have different levels of risk associated
- Competitive level. There's a big difference between social sport and elite level sport.
- Training Requirements / Workload. The higher the workload the more stress there is on the body
- Is there any protective equipment available to mitigate risk?
- Are there rule modifications related to the level or age of the participants?

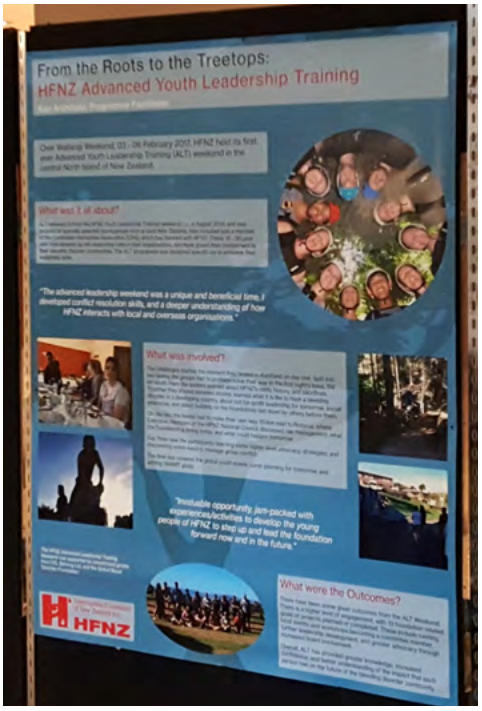
Finally, they factor in any risk tolerance modifiers: anything that makes the health risk more or less acceptable. These may include:

- The implications of any injury and its potential seriousness
- The impact on the participant's psychological wellbeing of being, or not being, involved in the activity
- Any socioeconomic factors

If the risk assessment exceeds the risk tolerance then the activity should be avoided. Due consideration should be given to short and long-term outcomes, and the potential for serious injury, like a head bleed. Also, look at whether there are factors that can be manipulated to further reduce the risk?

- Timing of Prophylaxis
- Compliance / Adherence
- Individual training program
- Coach education
- Athlete education

The take home message was that participating in sports is not a one-size-fits-all proposition, individualisation is the key, and decisions need to be made on a case-by-case basis. It's primarily important to understand the exact nature of the sport you wish to participate in, and the risks it actually presents. There must also be HTC involvement in the decision making process.



HFNZ Poster at ANZ Conference

Women and telling others

BY LYNNE CAMPBELL

This panel discussion session had a strong Australian bias as the speakers were talking primarily about disclosure and its implications as it pertained to navigating employment, buying and claiming insurance within the parameters of the Australian Insurance policies, Life insurance, Insurance Contracts legislation, and Disability Law Protection.

Historic Australian court cases were cited relating to the historic rights of an individual as they influenced legislation in Australia today.

In the panel discussion that followed it was clear that statistically fewer people within the general population had a bleeding disorder than not, so the "risk" to insurers was low.

If applying for Life or Travel Insurance one should always disclose one's bleeding disorder.

When seeking employment it is not compulsory to disclose unless your condition prevents you from doing the work you have been employed to do, or whether your safety and the safety of others is impacted as a result of you taking up that position. Ideally, prove you can do the job first.

Several people described their personal experience of disclosure in relationships and in employment. Some felt it was better to have an advocate with them. All felt it was necessary in dental and other surgical situations. All had (after initial reservation in some panel members) found it proved worthwhile in terms of raising awareness of their specific bleeding disorder and gaining

specific necessary support in their work places once their medical situation was known to colleagues.

Free Papers Session

BY NICKY HOLLINGS

Robyn Shoemark: To vein or not to vein

For people with severe haemophilia, prophylaxis has been the treatment of choice for many years. Haemophilia A is usually treated three times per week, or every second day. Haemophilia B is usually twice a week, or every three days. Peripheral venous access or a surgically implanted venous device (a port) are the most common ways to administer factor. Deciding what type of venous access is best is dependent on many variables, including age, size, vein health, and the frequency of treatment.

For children, starting prophylaxis often meant surgery to have a port inserted. With the advent of extended half-life products peripheral venous access has become a more viable option. Suitability for venous access can be linked to the size of their veins, location, previous cannula placement and number, treatment and focus, parent dedication. It's important to support parents to build rituals and distractions help with the normalisation and acceptance of treatment for child and parent.

We heard a story of a family at Sydney children's hospital who were willing to try venous access with their 14 month old, rather than have a port inserted. Their proximity to the hospital, and other family circumstances, made this a viable option, which. The HTC supported this choice, and taught them how to do the venous access. They used distraction, tv, surprise box, and played with toys after treatment. The child was 19 months old when dad had his first attempt by himself, which was successful.

In the beginning there were good and bad days, consistency was the key, same seat, same person, same vein. There were problems grumpy, wiggly, tired, hungry days, not hydrated or warm enough, needle dislodgement, difficulties holding the hand, days off, and holidays. However, today they are traveling and camping as a family. It has given them freedom to move, and also freedom for this little person to not be constantly at the hospital.

The new extended half-life products, willing parents and children, and consistent nursing staff offering appropriate levels of support may contribute to less need for ports for the treatment of children with haemophilia.

Jane Portnoy and Alex Coombs: A new transition model from the adult hospital perspective

Transition is well known for being a risky time for young people. The costs of unsuccessful transition extend across a young person's personal, family, financial, and health spheres. Adolescence is a time when there are physical and neurological changes, and we now know that at 18 the brain is still not fully developed, particularly when it comes to dealing with emotions, and how to blend them with reasoning and judgement.

Running a transition programme is about trying to build a relationship with our future adult clients, and reducing the challenges of having to disrupt healthcare at a time when there is so much else going on. There are many changes going on at this time, across many aspects of a young person's life, often all at once.

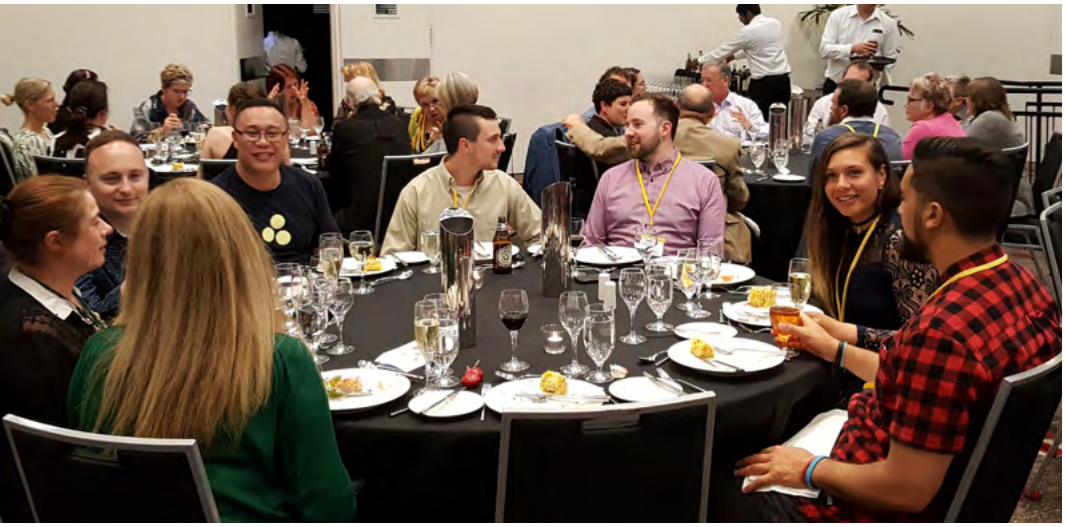
This project began with Jane and Alex noticing a group of their clients that really struggled with managing their own care. The impact is seen in the quality of their lives. It can lead to long-term harm and to significant feelings about coming into an adult hospital.

They decided that they needed to get to know these patients before they were flung into the adult system. To do this they had a variety of strategies:

- They ran an info evening, personally contacting all of their imminent transitioners
- They involved Haemophilia Australia and Haemophilia Victoria, contributing to their magazines and newsletters, and met with social workers, psychologists, and others working in the area of bleeding disorders
- They talked to recent transitioners about what worked and what didn't, particularly those who had had specific issues with their bleeding disorder, or in a hospital setting.

Dr Julie Curtain: Real benefits of new therapies for children with haemophilia

Even though there have been very good treatments available for the last 20 years, there are still a number of significant challenges associated with managing children with haemophilia. Recently a number of new products have become available that show great promise in addressing these issues. While, a cure for haemophilia remains the Holy Grail, progress on gene therapy looks as though the Grail may become reality. However, it is likely to still be quite some time



The HFNZ table at the conference dinner

before gene therapy becomes the regular treatment haemophilia patients. In the meantime there are a number of new products becoming available that extend the treatment half-life, work through alternative mechanisms, or are delivered by alternate means. These products are at different stages of testing or production in different parts of the world.

New Zealand is on a similar path as Australia. We have children in New Zealand who are on trials for long acting treatment for Haemophilia B. In Australia there are children that are on trials for emicizumab. Unfortunately, in New Zealand this is yet to become available.

Common current challenges in managing children with haemophilia include the need to administer treatment intravenously, the development of inhibitors, traumatic bleeds in active children, and issues with adherence to treatment schedules (especially in adolescents). The novel treatments that are now emerging may provide means to overcome these challenges. The benefits of these treatments include reduced frequency of infusion, reduced need for ports, improved trough levels, and treatments like emicizumab that work despite the presence of an inhibitor. Perhaps most importantly, the ability to deliver some of these products subcutaneously offers real promise to parents of young children, where finding a vein can be a real trial for all.

The new wave of novel therapies have the potential to revolutionise the treatment of children with haemophilia.

What's Here Now and on the Horizon

BY KARL ARCHIBALD

This session was an investigation of what is on the horizon for new treatment therapies. We heard from Paul, a person with haemophilia, about it how different his life is now that he is on an extended half-life product. There was an address by John Cahill, General Manager of the Australian National Blood Authority. Dr Huyen Tran talked about extended half-life therapies and other new treatments. Finally, Prof John Rasko shared exciting news about what's new in gene therapy.

Paul has been on Alprolix, an extended half-life, long-acting FIX treatment, for the last 5 years.

Before that, he was treating on demand. Because of his haphazard treatment, he has developed target joints and now has a fused left ankle.

He currently treats with Alprolix once a week. The change to a new product was seamless; his biggest challenge was the transition to a fixed routine for treatment.

Paul has not had a bleed since he started regularly dosing with the long-acting factor. His dosage has decreased from 6000 IU to 4000 IU per treatment, without compromising his health.

The biggest change for Paul is that he now has a more positive mind-set, and an improved quality of life. Paul now feels like he is 'normal' and his personal self-confidence has increased.

Next up was John Cahill, from the National Blood Authority (NBA) of Australia. The NBA is the statutory authority that controls all blood and blood product supplies in Australia, including products for the treatment of bleeding disorders. Much like NZ, Australia has a tender process. However, their tender lasts five years, rather than three like ours.

John's view is that it is essential to understand the importance of bleeding disorders, and of what the NBA's role is within the BD community.

He also recognised the need to look ahead to the prospect of funding and supplying the new and improved longer-acting therapies. John spoke about the processes and inputs of a multi-jurisdictional process, that it's important to be able to lobby the government, and to have a humanistic approach to these conversations.

Dr Huyen Tran followed, to talk about extended half-life factors and other new therapies. His presentation was from a researcher's perspective, and focussed on the current challenges and successes that are being made in the coagulation process.

Dr Tran reminded us that the key outcome researchers are always looking for is to get blood to clot in people with bleeding disorders. The key challenge, however, is how to do it effectively and efficiently.

When it comes to maintaining higher trough levels over a half-life, the challenge is to ensure adherence to prophylaxis over a lifetime to minimise arthropathy.

His team is having success with prophylaxis using extended half-life products in the reduction of bleeds in FIX patents, while treating in 7,14 and 18 day processes. Outcomes are also improving for FVIII patients.

For people with inhibitors and haemophilia B, dosed subcutaneously with emicizumab at <1% body weight once per week, there has been a 87% reduction in bleeding frequency, and a consequential 20% base-line clotting factor level; a 19% increase in bottom line. Two thirds of patients have had no bleeds since starting the treatment.

This is a potential life changer.

However, there are risks. The biggest is the mind-set change, to which Paul alluded. Sometimes, people trialling emicizumab, who did have a bleeding incident, would habitually treat in the same way that they always had, by taking a measured dose of their regular treatment product. This resulted in too much clotting factor in their blood, and the development of blood clots.

Our final speaker was Prof John Rasko, who spoke about some very exciting advances in gene therapy.

Gene therapy started from a very simple idea: One shot, and you're cured. The end. He thinks that it is really positive to see the development that this simple idea is making. However, this simple idea is very difficult to achieve.

We now have more than 10 years of safety data on gene therapy in haemophilia, and the watershed time is now. Gene therapy in the last weeks has been coming of age.

The FDA approved CAR-T cells for cancer treatment at the beginning of the year, RPEG5, for blindness, was approved by the FDA in October, and immunodeficiency gene therapy has now been approved for therapeutic use.

Currently, as a result of trials and treatment, over 10,000 people on this planet have now been gene modified.

Building off this, while all the work done to extend the half-life of factor replacement products is fantastic, gene therapy is showing the potential to maintain trough levels at or above 12% as the new normal.

So, how do we achieve this? We started with a viral vector, a common virus that we have been exposed to in early life, take out the bad payload, the bugs, then attach the missing gene, and put it back in.

However, while this sounds simple enough, there have been ongoing issues. During the first phase, there were sustained increased levels that lasted for 2 weeks. Then, they found that the immune system started destroying the carrier the virus, creating a negative outcome. It turned out that they were unable to use virus carriers that the patient had already been exposed to.

Building off this, we began to administer the virus in conjunction with steroids. The problem with that is that the patient is then stuck with the long-term use of steroids, effectively swapping one medication for another.

So now, we have a new process. We have a bio-engineered vector capsid and expression cassette, an artificial virus that no patient has ever been exposed to, which we can administer at low doses.

The outcome: In trials, infusing 100ml over 30 minutes, one single injection to a patient with severe FIX deficiency, resulted in a base line of 33% that has been consistent for two years. A Victorian patent has a consistent base line of 78% and another at 52%, although over shorter periods.

It is great to see these, very promising, results; however, there are still questions that need answering, and more work to be done. Prof Rasko feels that once this is complete it will be a good time for him to retire.

HFNZ AGM

2017 Adult Weekend and HFNZ AGM

On Friday September 29, members from across NZ gathered at the Ibis/Novotel in Ellerslie, Auckland for the 2017 Adult Weekend. This year we also added the HFNZ AGM into the mix. The people attending were looking forward to a weekend of new information, re-connection, enthusiasm, and FUN! Luckily, we had magic, sideshows, a quiz, guest speakers, and plenty of good food!

This year, as well as the usual mix of learning and socialising, we were celebrating the contribution of two long serving staff members, Colleen McKay and Leanne Pearce. That meant a few speeches, a few tears, and a whole lot of laughter. **Phil Constable** was there...

On Friday September 29 2017, members and their partners from around New Zealand made their way to Auckland for the 2017 Adult Weekend. In a break from tradition, we also ran the 2017 AGM as an add-on to the weekend, on Sunday October 1.

A big part of this weekend was allowing the members to have the opportunity to touch base with two of our long-serving staff members, who moved on to new things during the year. Colleen McKay and Leanne Pearce had, between them, over 32 years of experience working with our people as key members of the HFNZ team. This was a great chance to give them the send-off they deserved.

Attendees started drifting in to the Quality Hotel Parnell throughout the afternoon, so they could be present for the official welcome in the evening. The Richards, Scott and Chambers, said a few words, and everyone had a chance to mix and mingle socially, while enjoying a 'light meal'. Such was the excellent quality and quantity of the food, that the term 'light meal' came to be a bit of a running joke as the weekend continued.

A big part of this weekend was allowing the members to have the opportunity to touch base with two of our long-serving staff members, who moved on to new things during the year. Colleen McKay and Leanne Pearce had, between them, over 32 years of experience working with our people as key members of the HFNZ team. This was a great chance to give them the send-off they deserved.

Bright and early on Saturday morning we gathered for the first proper session of the weekend. We heard about the personal bleeding disorder journeys of Eric Kemsley, Nicola Cuthbert, and Tineke & Jono Maoate. Their stories were touching and often hard to hear, and clearly resonated with everyone present. Next, we heard from Dr. Karen Falloon, who spoke to us about the links between sleep and chronic pain. She had lots of very interesting information, and gave us plenty of tips about how to get the right amount of shut-eye each night. For the rest of the morning we split up into groups to talk about partner issues, men's issues, and women's issues.

Following a bite to eat and a cup of tea, we were treated to a clinical update from Maureen Hayes from the Waikato HTC. Maureen had plenty to say about new and upcoming treatments, particularly the rise and rise of ACE910, commonly known as emicizumab. It's really exciting to see all the new things on the horizon.

The remainder of the afternoon was given over to fun and games, as we took our first chance to recognise Colleen and Leanne. To start the afternoon off in style we enjoyed the skills of magician Thomas Hayman, who had some great tricks and a fine line in banter. Then, members had the chance to say a few words about how Colleen had influenced their lives. This was a very moving time, and several people shed a tear or two.

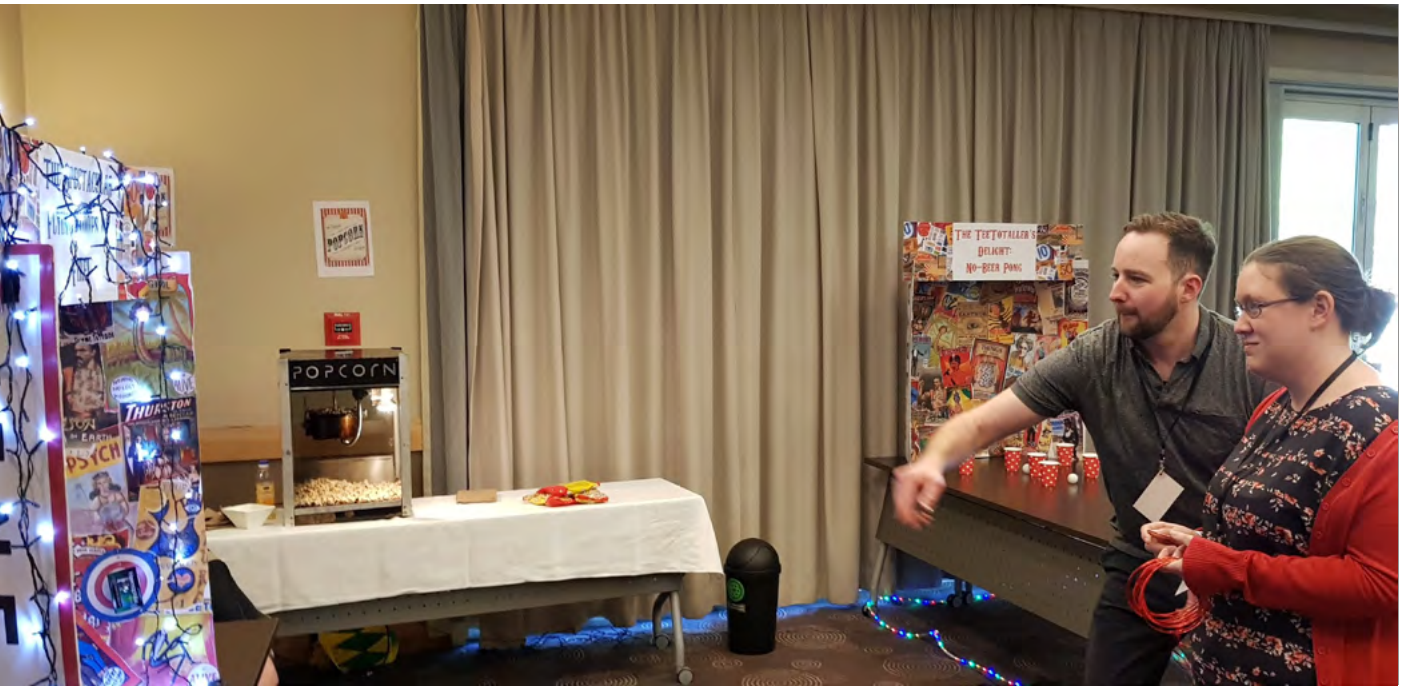
Next, we moved into the big HFNZ carnival. This was a great time, modelled on the vintage sideshows of old. There were some fantastic activities for people to get involved in, including Plucky Pearce's Precision Poultry Pitching and Camp Mother's Down-Home Antibody Shooting Party. A fantastic time was had by all, and it was a great opportunity for Colleen and Leanne to catch up with the members.

The Carnival merged into social time, before everyone gathered for the official dinner, and another opportunity to recognise Colleen and Leanne. Again, several people took the opportunity to say a few words, and Colleen spoke on behalf of herself and Leanne.

Dinner was followed by Quiz Time, where groups competed to prove just how smart they are when it comes to bleeding disorder facts, trivia, and general knowledge. There are some very clued-up people in our organisation, and the result was in the balance right up to the end of the night. Ultimately, Joy and Josiane's team snatched it at the end, to secure the coveted winners' medals.

The final sessions of Adult Weekend happened on Sunday morning. To start the day we had four quick-fire sessions from Hemi, who spoke about youth issues, Josiane, who talked employment, John Tuck, who extolled the virtues of ACE910, and Te Whainoa and Tuatahi, who talked about their experiences as Māori with bleeding disorders. These were very interesting sessions. It was particularly gratifying to hear just how much difference the ACE910 trial has made to John's life. A pretty amazing treatment!

The final session of the weekend was a talk by our special guest, Barry de Geest. Barry is a thalidomide child, he has very short, and minimally effective arms and legs. While he was able to walk as a younger man, Barry is now mostly wheelchair-bound. However, he certainly hasn't let his disability get in the way of his life. The title of his presentation was 'No Cotton Wool for Me!', and he has very much lived up to that statement. The stories he told of his achievements and challenges was inspirational, and directly applicable to our people. It was a real treat to listen to him.



Barry's session marked the end of Adult Weekend.

After a break for lunch, we moved on to the 2017 HFNZ Annual General Meeting. In Deon's absence, the AGM was co-chaired by HFNZ vice-presidents Catriona Gordon and Richard Scott. We had a very good turnout, largely due to of tagging the AGM on to the end of Adult Weekend. Several people who had not attended Adult Weekend also chose to come specifically for the AGM.

Decisions of note included long serving vice-president Richard Scott stepping down, to be replaced by Karl Archibald. Richard's service to HFNZ was noted and appreciated. We also had Neil Smith replacing Liam Brodie on National Council, and Karl Archibald and Blair Wightman taking up new roles on their respective regional committees.

The most significant piece of business was the approval of the Council's ability to borrow money from the Allan Coster Educational Endowment Trust, and from a trading bank or angel investor, to put towards buying a new building in Wellington. This new building will be the new HFNZ head office.

The AGM was concluded swiftly, and members enjoyed some afternoon tea, before heading towards home.

Overall, the whole weekend went very well. Everyone seemed to enjoy the educational and social activities at adult weekend, and the AGM was well attended.

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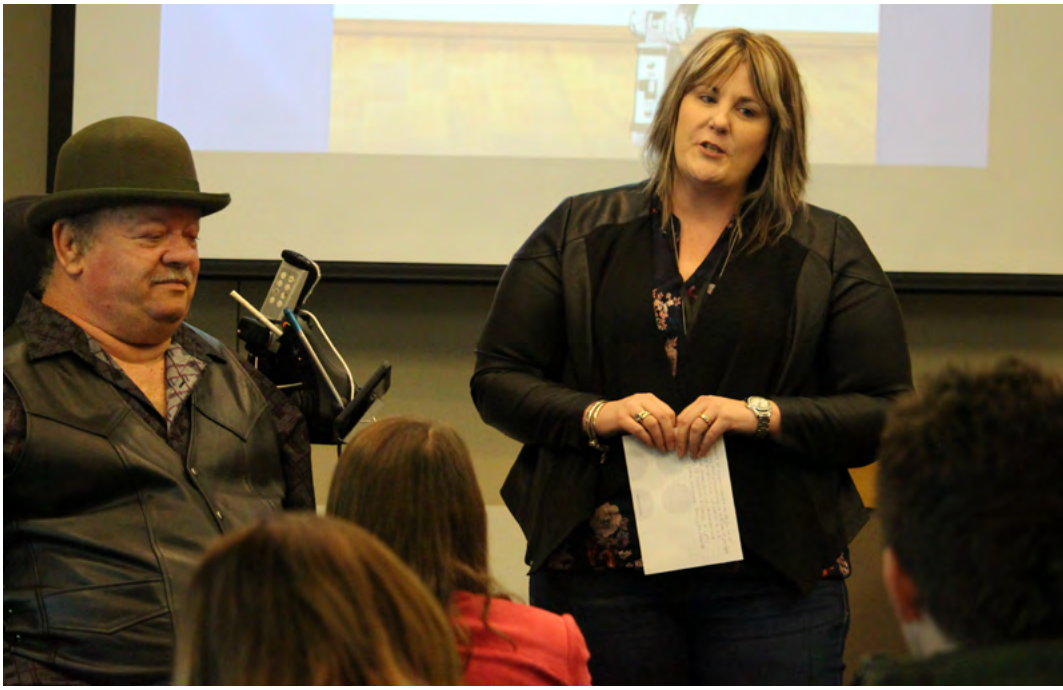

Farewell Josiane, welcome Ali...

For the last year, almost exactly, our Southern members have been lucky enough to have Josiane McGregor as their Outreach Worker. Unfortunately, Josiane's time at HFNZ has ended, so we must wish her farewell, and safe travels.

Josiane came to us from several years at Workbridge, and all that expertise came with her, to the benefit of us all. She made some great connections with the people in her region, and contributed in other areas too. She became the chief contributor to the regular PEPtalk section of our Pānui newsletter, and added plenty to the events and workshops she attended. Along the way, she took every opportunity to add her wide knowledge of employment and job seeking into the mix. She led sessions at various events, and submitted an extensive article to Bloodline highlighting some of the key things for our people to keep in mind when seeking employment.

Josiane also made her mark with her bone-dry sense of humour, and her ability to cut through the fluff and get to the heart of a matter. These are valuable traits in any workplace, and we will miss them. Josiane has taken on a role at the Tertiary Education Commission in their Careers Directorate, as a Consultant for Migrant Futures. Sounds flash! Our loss is their gain.

Following Josiane's resignation, the head office team swung quickly into action to appoint her successor. After a thorough process, during which we interviewed several very well qualified and experienced candidates, we are delighted to welcome Ali Mitchell into the HFNZ whānau.

Ali comes us by way of Scotland, with a wealth of experience as a nurse in clinical and research settings. Most recently, she's been doing research with a team out of Otago University, and has worked in an outreach type role for the NZ Cancer Society.

Ali has a wide range of skills and interests, not the least of which is chocolate making, that we know will see her in good stead as she gets to grips with her new role. I know that everyone in the Southern region, and across the HFNZ whānau, will welcome her warmly, and help to make her transition a smooth one.

Ali's first day was Monday November 20th. You can contact her at ali@haemophilia.org.nz, or by calling Josiane's old number, 021 656 804.

Images top and bottom:
Josiane thanks Barry de Geest.
Ali, the new Southern Outreach Worker

Youth Twinning: Nepal

Earlier this year the HFNZ National Youth Committee (NYC) were invited to submit an application to twin with another youth organisation from a developing nation. The nation they were paired with was Nepal. The initial application involved sending two members to Nepal to meet with local members, and officials, write a report, and submit it to WFH for consideration.

Our representatives for this first visit were **Hemirau Waretini** and **Courtney Stevens**. They made a short sharp visit to Nepal in early October.

Over the last several years HFNZ have been a part of the WFH Twinning programme. Twinning is where an organisation from a developed nation pairs up with one from a less developed nation, with a view to improving systems and processes in the developing nation. Most recently, HFNZ collaborated with CHA, the Cambodian Hemophilia Association. This was a very successful partnership, which resulted in great gains for both organisations.

As a direct result of this successful twinning, HFNZ were invited to apply to participate in a Youth twinning programme. In this version of twinning, our Youth group connects with the youth group of a developing organisation, to try to improve the way they operate. The country selected for us to pair up with is Nepal. The first step is for some of our people to visit Nepal, meet with some of the key people there, and complete the detailed application based on this visit.

From October 5 to 7, we made a flying visit to Nepal to get the twinning ball rolling. This really was a quick visit, but it involved a whole lot in a very short space of time.

The first day of our visit was full of meetings and greetings.

Sachi Satapathy talked to us all about WFH and the programmes and initiative they have in place to help develop the world bleeding disorder community. The Nepal Hemophilia Society representative talked about the NHS, where it came from, what it does, whom it works with, and how it's organised. He also spoke about some of the goals and aspirations of the Society. We also heard from the NHS youth committee, about who they are, what they do, and what their plans are. Finally, we gave a presentation about HFNZ and the National Youth Committee (NYC).

At the end of the day, in a welcome break from all the talking and listening, we visited the beautiful Pashupatinath temple

On the second day, we began by visiting the Bir Hospital. The hospital provides the area used for haemophilia patients, and the NHS funds the nurses. Four nurses work six-hour shifts six days a week, to provide medical support to people with haemophilia.

This HTC has been in existence for approximately 20 years, and has been open 24/7 since the beginning of 2017 (10 months). The HTC has four dedicated beds, and sees 10-15 patients per day. Plant and equipment, like fridges and freezers are donated. There is one freezer containing plasma in the HTC. There is a pallet for nurses working the night shift to nap on. The HTC is on the first floor with no elevator. Patients need to walk up the stairs to get there.

The nurses keep daily logs of patients, their details, the amount of factor administered, and the number of times they have visited the HTC. They also keep patient logbooks with details of each of the patient's visits and their 'prescriptions'. Patients will come in, have a treatment, and leave once the nurse thinks they are okay. Patients can self-infuse if they know how.

In Nepal, a doctor is necessary to prescribe medication. As there is no haematologist at Bir Hospital, the doctor on duty will be called to prescribe any treatment required. The reason for a doctor's sign-off is that the doctor 'knows' how to establish whether a treatment is necessary (as indicated by the level of pain that the patient is in).

Sometimes the HTC nurses may disagree with a doctor who is reluctant to prescribe medication because they don't believe that the patient is in enough pain. In this situation, the nurses advocate on behalf of their patient to receive treatment.

The only factor that they have comes in large doses. As they limit treatment due to supply, they often split vials between patients; giving half to one patient and returning the remainder to the fridge (for use within 3 hours). The NHS holds the treatment, and supplies sent to HTC when a top-up of the stock is required.

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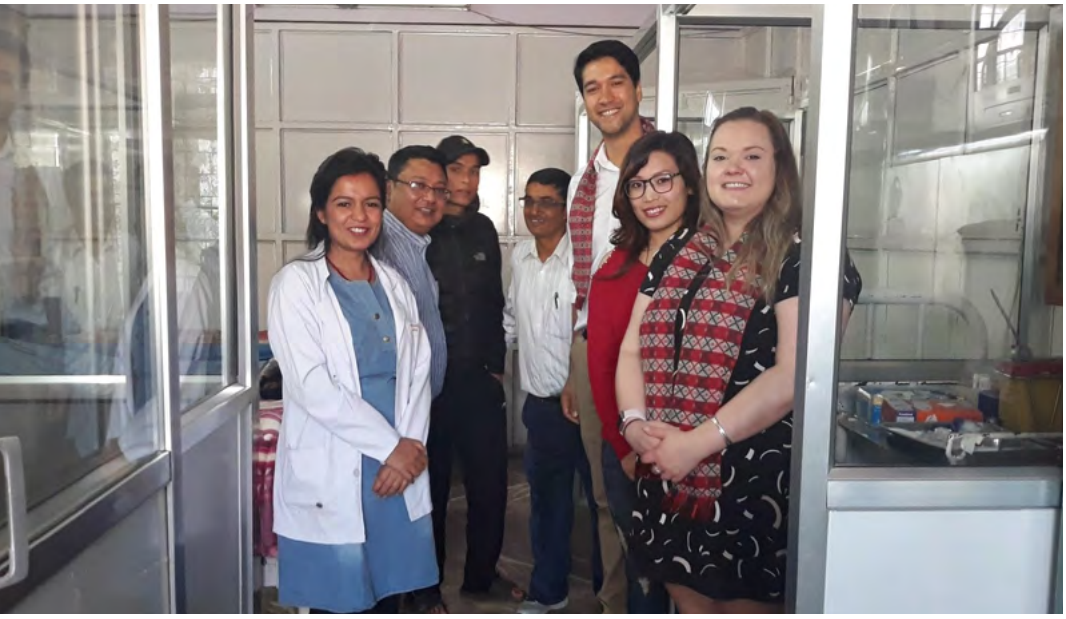
There is no dedicated haemophilia physiotherapist at Bir Hospital. However, there is a regular physio, to whom the nurses refer mild patients with minor bleeds and issues. There is also a geneticist at Bir Hospital, who is very keen to start PGD screening but lacks the funding for even a pilot study.

Next, we visited the Civil Hospital. This is a larger and newer hospital, and it is cleaner than Bir Hospital. This hospital is better equipped with diagnostic materials, and even has a machine that will extract only the required part of blood from donors (i.e. FIX, FVIII, platelets, vWF...)



Hospital visit, Nepal

When we arrived there was a line out the door, with about 400 people in it who were queueing to 'take a ticket' with a number, and then wait their turn to be called.

The HTC is part of the oncology ward, and the Haematologists also see the oncology patients. Haemophilia patients take up about 10-20% of their time. There are two Haematologists, two Haemophilia Nurses, one Geneticist, and one other doctor. There is a very motivated team in place, the head haematologist leads the discussions and outlines their plans.

They have no faith that the government will provide funding for treatment or improved care, and so have taken it upon themselves to set the direction to improve haemophilia treatment in Nepal.

One idea is to have training sessions with doctors in hospitals without HTCs to give general practitioners a 'crash course' in haemophilia identification and care. This will improve the general knowledge in a country where a major issue is the identification of bleeding disorders and the diagnosis of patients. WFH has agreed to provide experts to facilitate these workshops.

Later, we had a visit with the deputy director of the Nepal health service. He reports a good relationship with the NHS president and general secretary, and is interested in looking at improving healthcare around diagnosis. He also expressed interest in New Zealand's comprehensive care system, as well as a desire to attend the 2018 WFH World Congress in Glasgow.

At the end of the day, a mother of a child with haemophilia took us to Swayambhu temple, situated on a peak within the valley, which offered a panoramic view of the Kathmandu Valley. On our final day, we met with the youth committee again, and broke up into groups to determine whether HFNZ is in a position to help the NHS youth. We did a SWOT analysis (Strengths, Weaknesses, Opportunities, and Threats) to determine what approach would be best.

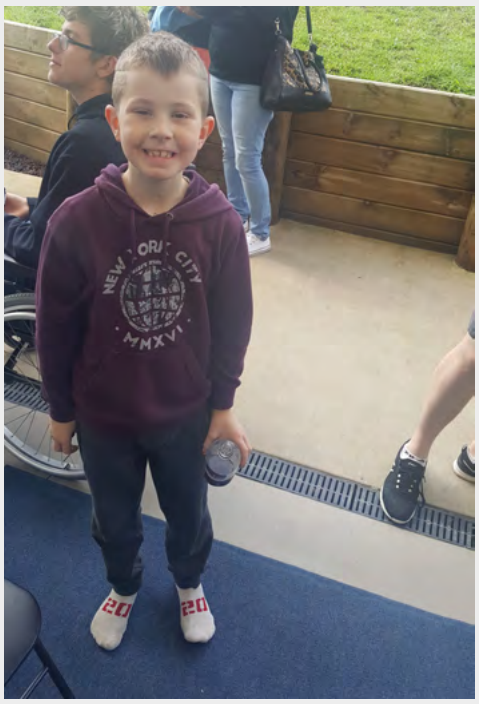
The youth of the NHS are particularly interested in leadership skills and motivational training. The areas of leadership they are most interested in include technical skills related to fundraising, and the continued development of the organisational structure of the NHS, and their youth committee.

One idea is to have training sessions with doctors in hospitals without HTCs to give general practitioners a 'crash course' in haemophilia identification and care. This will improve the general knowledge in a country where a major issue is the identification of bleeding disorders and the diagnosis of patients. WFH has agreed to provide experts to facilitate these workshops.

Since completing the trip, we have had our application approved. Now the real work begins...

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 as the weather improved and the holiday season loomed on the horizon.



Images top right:
Fun at Silverdale.

Central Report – Nov 2017

By Blair Wightman

As a long-time member of the Foundation, I'm excited to take over the role of Central Region Chairperson from Stephanie Coulman. I look forward to meeting many of you over the coming months.

In August, we had our Central AGM at Owlcatraz in Shannon. We completed the form-owl-aties before lunch, followed by an afternoon being treated to a tour of the Owls and countless Owl-puns by the Owlcatraz team.

We also welcome Ross Gordon into the Treasurer role, and farewelled Stephanie and Judith Dudson. Judith has stepped down after more than a decade on the committee, and Stephanie has done a fantastic job as our Chairperson since 2012. Stephanie continues on the committee as our Council delegate. Thanks to you both for the energy you have put into running many successful events!

Looking forward, we've got a busy summer period coming up for the central region – with a bit of a nautical theme! Our Christmas event is a cruise around Wellington harbour on the evening of 25 November followed by our men's fishing trip, which has been postponed until 27 January 2018.

The Christmas cruise is open for registration, so if you'd like to RSVP, please send us an email on hfnzcentral@gmail.com. We also have a small number of places left for the fishing trip, so let us know if you are interested. We'll be in touch with everyone who has already registered for the fishing trip shortly to confirm your availability.

The Central committee is here as the Foundation's local presence in the region, do let us know if you have any ideas for events/activities you'd like to see in the region. We'd also welcome anyone interested onto our committee.

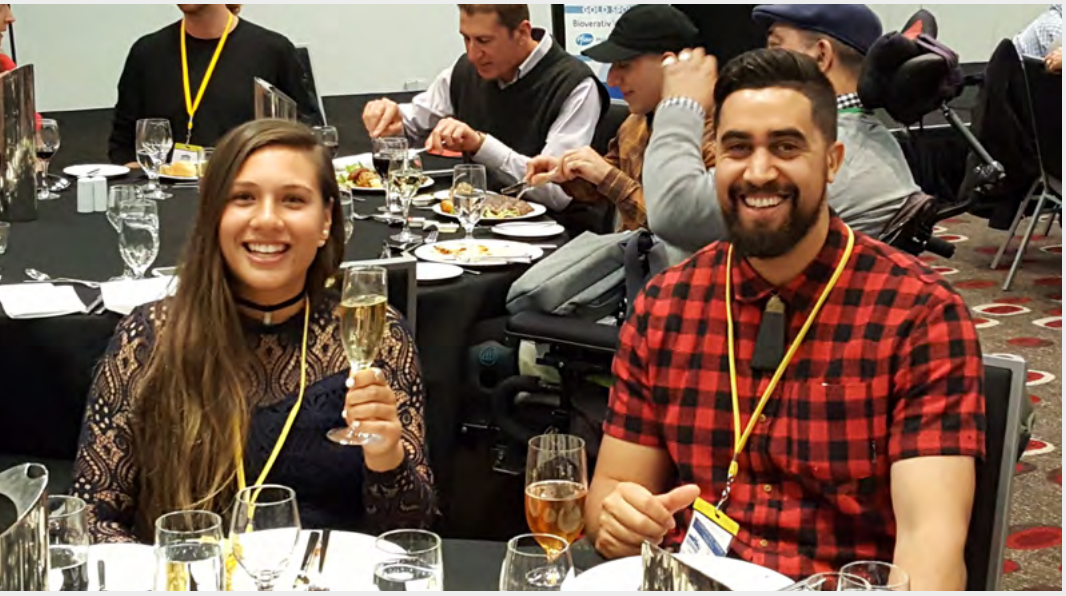
Northern

By Neil Smith

Hi all,

Our last event was a fantastic day out at Silverdale Adventure Park, and also doubled as the Northern AGM. It's a bit of a hidden gem out next to snowplanet north of Auckland, and there was a good bit of adult fun on the luge (with my back still recovering) and plenty of loud/flashing/spinning things for kids. At the AGM portion of the event, we farewelled Richard Scott who, as Chairman of the Northern Committee, has led us through thick and thin over many a year, and, along with Lynley, has basically kept the committee going (quite possibly since before I was born (jokes)!) Thanks Richard. Our thanks to Liam and Jess for their contributions too – really appreciated. Everything they have done is on behalf of our members to make the best use of the opportunities we have.

So out with the old, and welcome aboard to Dustin who joins Greg, Hemi, and I to get our events sorted for the next year. We don't have a Chair as such, so we've gone all MMP. As an awesome foursome, we are awesome, but thankfully we have Nicky to



help, and we'll need you all to lend a hand with some of the things we've got coming up. Which leads me to...next up we have our Christmas BBQ out at Parakai this year and the big red fella might make an appearance. Sunday 3rd December with a BBQ lunch so look out for invites and be sure to RSVP. Bring your tongs and thongs.

Further out, we've got our summer camp booked for 9th to 11th March, which is out Drury way (south of south Auckland) at a place called Chosen Valley. Last year was a cracker so I suggest you get in early as space can be limited. If you haven't got on board with the Northern HFNZ facebook group then please give Nicky a yell; it's a simple method for keeping you up to date with events, particularly when we don't have the right snail mail address for many of you.

Ideas for events for now up to July next year would be much appreciated. Just give us a yell.See you at Parakai, I hope.

Piritoto: My experience at the Australian & New Zealand Conference on Haemophilia & Rare Bleeding Disorders

By Mahia Nightingale-Pene

I was lucky enough to attend the Australian & New Zealand Conference on Haemophilia & Rare Bleeding Disorders. There were so many things that blew my mind throughout the 3 days that made me very grateful to have been there. The food was amazing, the knowledge from the professionals was influential, the topics were all relevant and informative, but the thing that makes me grateful for the most - is the people.

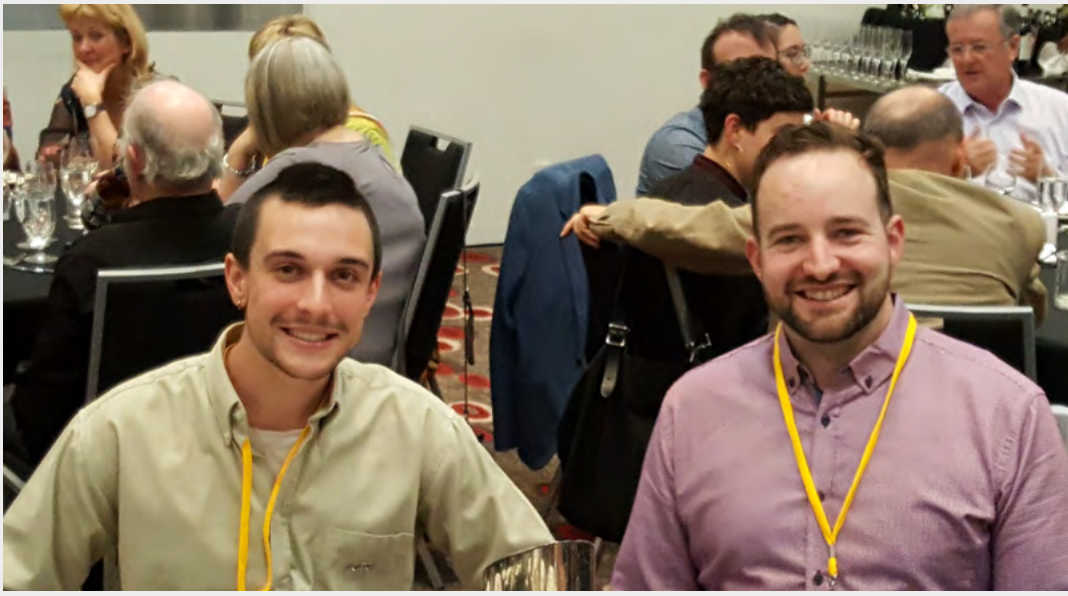
The very first lot of people I spoke with was my fellow New Zealanders. It was awesome to meet and be amongst the NZ crew. Finally meeting them was a relief because I found myself relaxed. Deon, Karl, Phil, Nicky, Lynne, and Zac; it was great meeting you all and getting to know you. You guys are awesome! Thank you for being another piece of home for me.

At the event there were pop-up booths with information pamphlets and books, plenty of chocolate, and people who were passionate about creating resources that benefited people with bleeding disorders. I now have many resources from those lovely people that I often read.

I was lucky enough to have befriended two people from Australia –Lexi and Patrick. Lexi has the gift of the gab and is effortlessly hilarious; Patrick is a very tall lad with a fair amount of knowledge about NZ that had us conversing quite a bit about where we both are from. Having friends at the conference made it that much more enjoyable.

The conference was jam-packed with korero from special speakers. The speakers spoke about their knowledge that I found worth more than gold. Although some information was overwhelming, the topics were relevant to m and I am grateful to have learnt so much. I now have a notebook with loads of valuable information.

There were also special moments that really touched my heart. I had a moment with two mothers from Australia at the Women's Breakfast. One mother spoke to us about the moment she found out her son has Haemophilia. She told the story so well that I could imagine her house, her new-born baby, the hospital, her car, and the other people involved – absolutely everything. I was really moved and grateful to hear her story.



Another special moment for me was re-meeting Colleen. The last time she and I saw each other was when I was a wee girl. She spoke about how much I have grown, my whānau, and my late Nana – which brought us both to tears. That moment was the most outstanding moment for me and I am grateful to have had that moment with her.

Overall, I am very grateful. Grateful to have the opportunity to be there, grateful for all the great knowledge I learnt, but most of all I am grateful for all the people I met. Meeting those people made the conference even greater.

Mokori anō kia mihi ki a koutou i whakaae ki a haere atu māua ko taku ipo ki tēnei hui taumata motuhake. Kei a koutou mo te manaaki me te whai whakaaro nui ki te tangata.

Nei aku mihi,

Māhia

Southern

By Karl Archibald

Since the last edition of Bloodline, the Southern Region has gone through a period of transition.We have unfortunately lost a valued outreach worker, Josiane McGregor, who has taken on a new and exciting opportunity.

On behalf of Southern, I would like to thank her for all of her hard work, support, advice, and knowledge that she has shared and empowered members of Southern and HFNZ with over the last 11 months.

However, where one valued staff member departs, another is welcomed. Welcome to Ali Mitchel. As mentioned in the latest Pānui, Ali comes from a nursing, project management and health promotion background.

In addition, she was brought up in Scotland, so I have no doubt that she will fit right in to Highlanders country, as well as with the rest of the region. We look forward to her coming on board.There has also been a couple of changes within the regional committee.

Below are the Southern committee members and office holders.

- Southern Youth Delegate: Zac Porter
- Southern Delegate to Council: Theresa Stevens
- Secretary: Susan Inwood
- Committee Members:
  - Paul and Leanne Spencer
  - Heather Phillips

And myself, Karl Archibald, as Chair.

I would like to thank the previous, continuing, and new volunteers for their ongoing support and dedication. Without them events such as the Southern Regional Christmas party, on Sunday the 10th of December 2017, would not be possible. - Keep an eye out for further information.

Images top left and right:

Mahia & Hakopa at the ANZ Conference.

Zac & Karl at the ANZ Conference.

News from around the world

Enrollment Begins in Study Evaluating Immune Tolerance Induction with Elocta

MATHEW SHANLEY

Swedish Orphan Biovitrum AB (Sobi) announced this morning that the first patient was enrolled in the open-label, multicenter RelTirate study (NCT03103542) designed to test Elocta (efmoroctocog alfa) in patients with hemophilia A. The trial is a collaborative effort with Bioverativ Therapuetics Inc.

The study is examining the immune tolerance induction (ITI) potential of Elocta in hemophilia A patients who have developed inhibitors that have failed to be resolved with other therapies.

Hemophilia A is characterized by a patient's inability to properly form blood clots as the result of missing or reduced levels of the Factor VIII protein. The rare genetic disorder leads to patients experiencing recurrent painful bleeding episodes, some of which can be life-threatening. The current standard of care for hemophilia A is replacement therapy, however, some patients develop antibodies that can make that therapy ineffective.

"We are committed to provide treatment options to patients with hemophilia that will enable them to make choices and live the lives they would like to live. The ReITIrate study is very much aligned with that commitment and may address one of the most critical questions – treatment management of patients who have developed inhibitors," says Krassimir Mitchev, MD, PhD, Vice President and Medical Therapeutic Area Head of Haemophilia at Sobi in a press release.

The safety and efficacy of Elocta in previously-treated patients have both already been proven in clinical trials and further confirmed in real-world clinical settings. It is a recombinant clotting factor therapy developed using Fc fusion technology to prolong circulation in the body. It is engineered by fusing Factor VIII to the Fc portion of immunoglobulin G subclass 1 (IgG1), enabling Elocta to use an organically occurring pathway to lengthen the time the therapy remains in the body.

The partnership between Sobi and Bioverativ for Elocta includes the former's final development and commercialization rights in Europe, North Africa, Russia and most Middle Eastern markets. Bioverativ has all manufacturing responsibility and final development and commercialization rights in North America and all other regions in the world.

In November 2015, Elocta was approved in Europe for the treatment of hemophilia A.

Source: http://www.raredr.com/news/enrollment-begins-in-study-evaluating-immune-tolerance-induction-with-elocta

FDA Orphan Drug Designation Granted to Hemophilia Gene Therapy; Clinical Studies to Begin Soon

MATHEW SHANLEY

The U.S. Food and Drug Administration (FDA) has granted Shire with orphan drug designation for its gene therapy candidate SHP654, intended to treat hemophilia A. The drug includes technology acquired from Chatham Therapeutics, LLC, a spin-out of Asklepios Biopharmaceutical, Inc.

Gene therapy is predicated around the delivery of a functional copy of a defective gene. For hemophilia A specifically, the gene in question is FVIII. SHP654 is being studied as a potential treatment for patients with the condition via the delivery of a long-term, constant level of the coagulating factor VIII. The gene therapy candidate was a developed molecule from Baxter/Baxalta (where it was designated BAX 888). When Shire acquired Baxalta in June 2016, the molecule was renamed to SHP654.

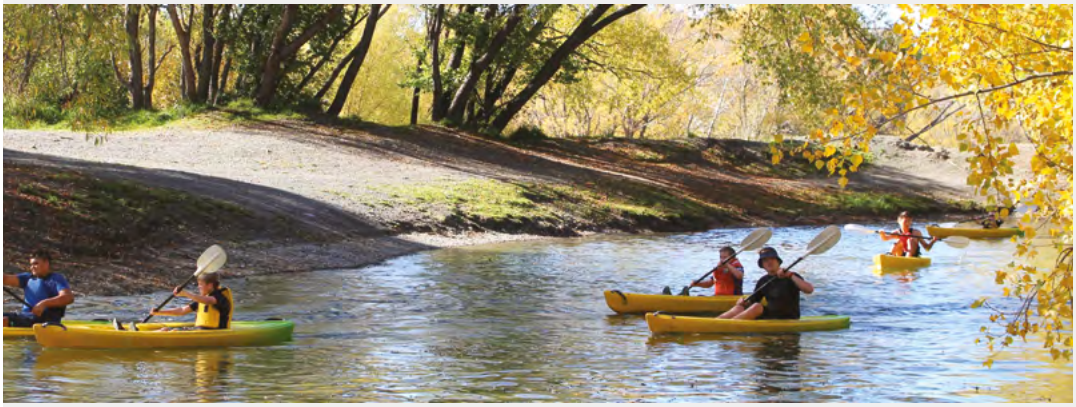
Hemophilia A is a genetic bleeding disorder and the most common type of hemophilia, affecting approximately 14,000 people in the United States. It is typically the result of patients not having enough factor VIII to combine factors IXa and X in the clotting cascade. Most treatments for hemophilia A consist of factor replacement therapy, but an estimated one-third of patients with hemophilia A develop inhibitors to standard factor VIII replacement therapies, limiting treatment options.

SHP654 uses an adeno-associated virus stereotype (AAV8) vector to deliver a codon-optimized, B-domain deleted FVIII (BD-VIII) specifically to a patient's liver. There, FVIII would be manufactured and used to regulate bleeds. The primary goal of the potential therapy is to provide a constant level of factor expression over several years and eliminate the inconsistency often associated with factor replacement therapy.

"This important Orphan Drug Designation highlights Shire's commitment to patients with rare diseases; and for hemophilia patients specifically our aim is to help them achieve zero bleeds," says Paul Monahan, M.D., Shire's Senior Medical Director of Gene Therapy in a press release. "We know that hemophilia care is not one-size-fits-all and that every patient is unique, which is why we continue to focus on optimizing personal outcomes for hemophilia patients by developing innovations to transform care."

In addition to the orphan drug designation, the FDA also awarded Shire an investigational new drug (IND) status for the potential therapy earlier this year. A phase 1/2 study is expected to begin in the final quarter of 2017.

Source: http://www.raredr.com/news/shp654-orphan-drug-designation



The Year Ahead...

More details on all events are available from your Outreach Worker

January 19-21, 2018

- **North American Camping Conference for Hemophilia Organizations (NACCHO)**
  Arizona, USA.

February 17 & 18, 2018

- **Para Kore Marae Noho, Raglan**
  Venue to be confirmed

April & July school holidays 2018

- **Regional Children's Workshops**
  Central & Midland in April; Northern & Southern in July.
  Final dates and venues tba

May 20-24, 2018

- **WFH World Congress**
  Glasgow, Scotland.

Late 2018

- **Women's Wellness Weekend**
  Date and venue tba

October 27 & 28, 2018

- **HFNZ AGM and 60th Birthday Celebration**
  Wellington, NZ. Date and venue tba

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

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