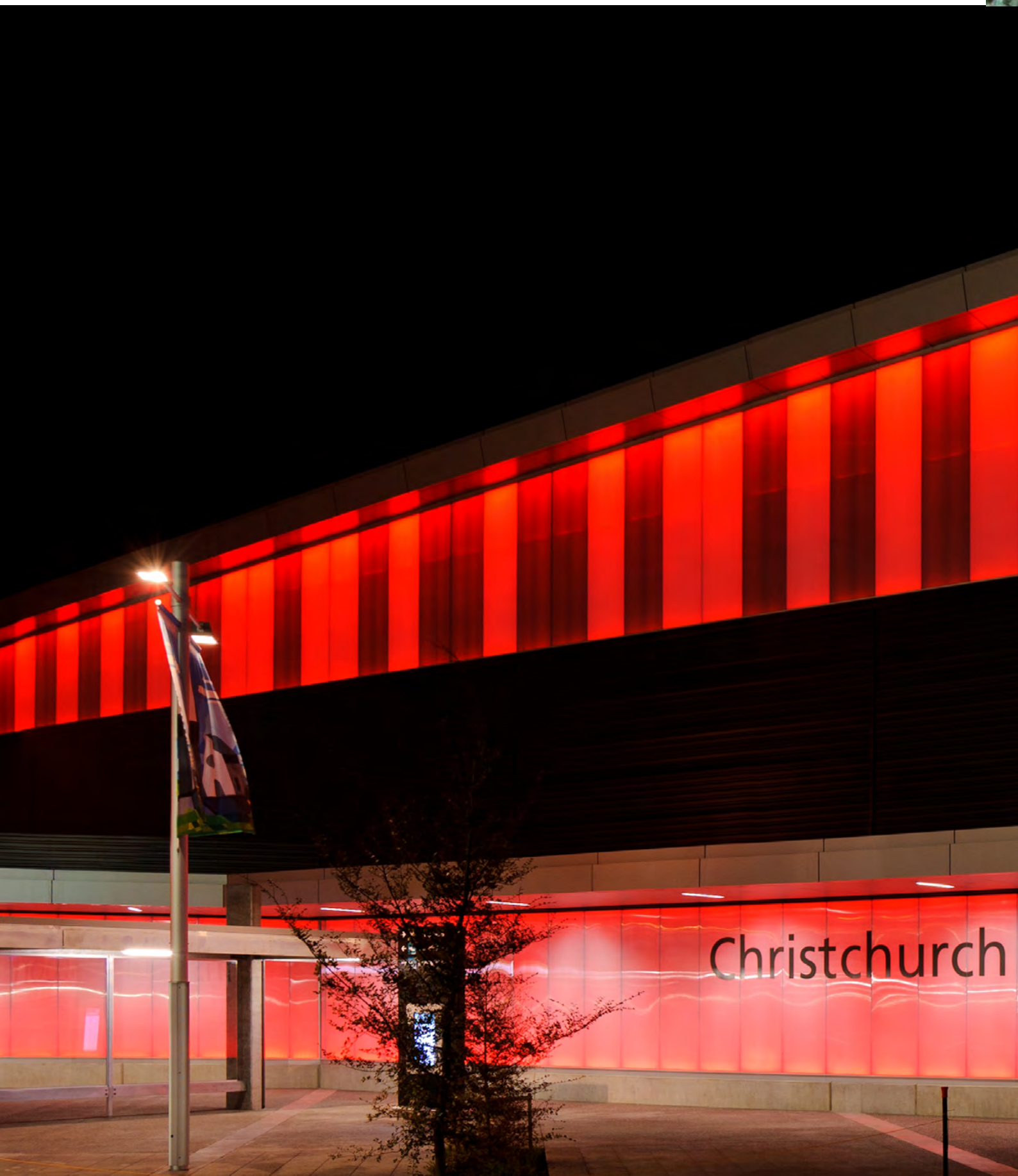
Bloodline

VOLUME 47. NUMBER 1
MARCH 2019

Haemophilia Foundation of New Zealand Inc.
HFNZ



Bloodline

Magazine of the Haemophilia Foundation of New Zealand. Volume 47 Number 1

CONTENTS

The purpose of this magazine is to provide a wide range of accurate and timely information on all aspects of haemophilia and related disorders. Haemophilia is a dynamic specialty and therefore opinion may change or be varied from time to time.

CONTACTS

Website

www.haemophilia.org.nz

National Office

PO Box 7647
Sydenham
Christchurch 8240
03 371 7477
info@haemophilia.org.nz

President

Deon York
president@haemophilia.org.nz

Chief Executive

Sue Ellis
sue@haemophilia.org.nz

Editor & Communications Manager

Phil Constable
phil@haemophilia.org.nz

Administrator

admin@haemophilia.org.nz
03 371 7477

Ross Paterson

Southern Outreach Worker
PO Box 7647, Sydenham
Christchurch 8240
03 371 7485
ross@haemophilia.org.nz

Lynne Campbell

Central Outreach Worker
PO Box 24014
Manners Street
Central Wellington 6142
04 382 8442
lynne@haemophilia.org.nz

Nicky Hollings

Midland Outreach Worker
PO Box 357
Taupo 3330
07 856 4442
nicky@haemophilia.org.nz

Amber Maihi

Northern Outreach Worker
PO Box 41-062
St Lukes, Mt Albert
Auckland 1346
09 845 4658
amber@haemophilia.org.nz

Haemophilia Foundation of New Zealand Inc.
HFNZ

OUTREACH FREEPHONE
0508 322 867
HAEMOPHILIANZ

Bank Details

Acct Name: Haemophilia Foundation of New Zealand
Acct Number: 02 0828 0102656 000

THE H WORD

PRODUCT CHANGES FROM 1 MAY 2019

From 1 May 2019, the funding of treatment for haemophilia will change. What does it mean for you, and what should you do? First, it's important to know that the proposed changes are encouraging: longer acting factor VIII and factor IX will both be funded. Next time you are at your appointment, discuss this with your team. If you are on propyhlaxis, or plan to be on propyhlaxis, you are eligble. There is no age cut off, this is for everyone.

In a time of transition, we have also noted that, along with treatment centres, we will need to provide training and education to our members on what it means to be on a longer-acting product, and how to draw up the new kit.

If there is a clinical reason you cannot change, then you will not need to switch products.

HFNZ agrees with the proposed changes. The following position was taken on your behalf:

The proposal to fund extended half-life rFVIII and rFIX is welcomed. This position is equitable for all people living with haemophilia in New Zealand provided that the plan for roll out of longer-acting therapies is consistently applied across all haemophilia treatment centres.

It is likely that most of our members (particularly those with severe haemophilia A and B) will want to change to extended half-life rFVIII and rFIX.

We do not have a strong preference over which longer-acting products are ultimately funded. We did, however, point out that the median half-life for products varies.

For patients with inhibitors, we note the continued funding of FEIBA and Novoseven. We would also highlight the potential of

Hemlibra (emicizumab) to radically change how haemophilia A is treated, both for patients with and without inhibitors.

PARTYING LIKE IT'S 2018.

Registration is open for the celebration of the first 60 years of HFNZ. We officially turned 60 on 13 May 2018, but we are saving the party to November 8 to 10 2019. For more information go to page 14 of Bloodline.

Deon York
HFNZ President

A Message from HFNZ

They were mothers, fathers, sons, and daughters. They were IT workers, and doctors, and parents, and students. They were important, and valuable, and loved. They were bound together by their faith, by their beliefs, and because they were a part of the community, the city, the New Zealand that we call home.

HFNZ expresses its heartbreak at this senseless taking of life, and its unequivocal support for the families and individuals affected by the events of March 15.

إِنَّا لِلَّهِ وَإِنَّـا إِلَيْهِ رَاجِعُونَ - inna lillahi wa inna ilayhi raji'un - we belong to Allah and to Allah we shall return. This is what Muslims say when they hear that someone has died. This will have been said a lot over the last weeks. Too often. The appalling terrorist attack on people worshipping at the Deans Ave and Linwood Ave mosques in Christchurch on March 15 brings into stark relief how important it is for us to be unified and inclusive.

When news first started coming through of a shooting in Christchurch, many of us would have dismissed it as another drive-by, or perhaps a robbery. None of us would have even considered the possibility of a racist tragedy on the scale that unfolded. That's just not what happens in NZ, we say. Except this sort of thing does happen.

Let this tragedy be a reminder of the price of separatism and discrimination, and a signpost for unity in the face of intolerance.

Bleeding disorders do not discriminate. HFNZ's membership includes and embraces a wide cross-section of New Zealand. We represent people who identify with a variety of ethnicity, of culture, of faith, of gender, of sexual orientation, and of socio-economic standing. We understand that diversity is what makes us whole. We know that inclusion, tolerance, and acceptance are the keys to peace, and to community. HFNZ has no room for hate.

This is reflected in the waiata adopted by HFNZ several years ago:

He hōnore, he korōria
Maungārongo ki te whenua
Whakaaro pai e
Kingā tangata katoa
Ake ake, ake ake

Honour, glory, and
peace to the land
May good thoughts come
to all people
for ever and ever, for ever and ever.

It is our hope and desire that the enormous outpouring of grief, support, and solidarity from all New Zealand is a catalyst for change. Terrorism is meant to breed fear and discord. These last few days have shown that this has no future in NZ. We have come together and shown that we are not so easily divided.

If you are feeling unduly affected by this tragedy, remember that our Outreach team are available for support. Just call 0508 FACTOR (322 867). Alternatively, there are several other groups in our community that can offer support:

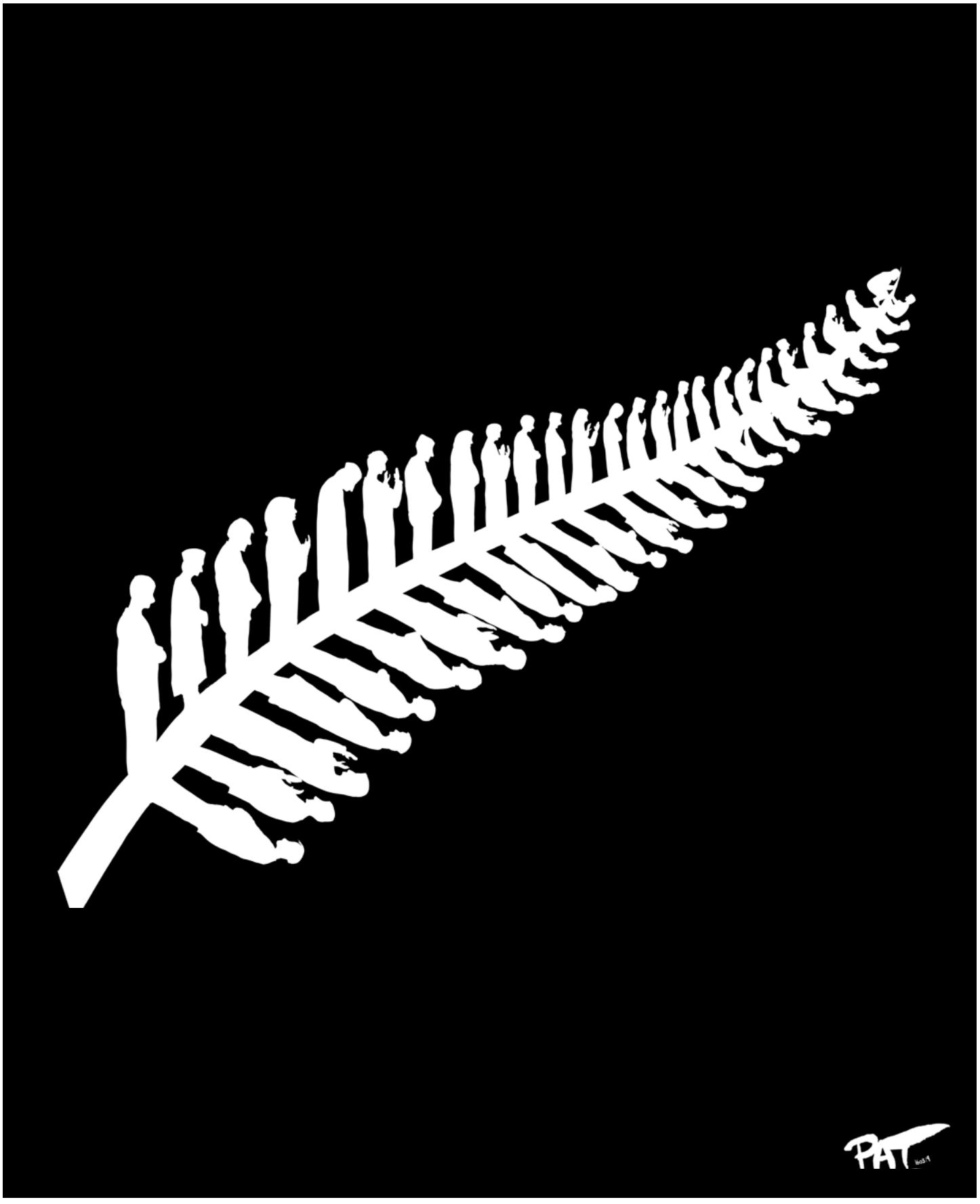
- **Lifeline: 0800 543 354**
- **Need To Talk: free text or call 1737**
- **Victim Support: 0800 842 846**

Waiho i te toipoto, kaua i te toiroa. Let us be close together, not far apart.

Ngā mihi nui,

The HFNZ Team.

Image credit: Pat Campbell and The Canberra Times, 2019


PAT

Rorie Poff's Australian Jamboree Adventure

BY RORIE AND SANDRA POFF

Over the summer, one of our young members went on a big adventure. Rorie Poff, a Southern member with severe haemophilia A, headed over to Australia to attend the Australian Scout Jamboree in Adelaide. We are very proud of the way Rorie was able to manage himself in a new and challenging environment. Here's his jamboree story.

Rorie is 13 years old and is a Scout at Lincoln Scout Group on the outskirts of Christchurch in New Zealand. Rorie has severe Haemophilia A. Over the summer, Rorie travelled with the New Zealand Scout contingent to the Australian jamboree. There were 55 scouts and 18 leaders from New Zealand for the trip. They spent a few days in Sydney at the Baden Powell Scout Park in Pennant Hills before heading over to Tailem Bend in Adelaide, where the jamboree site was.

During their time in Sydney, they were able to get out and about around the city, with visits to the Australian Museum, Manley Beach, Darling Harbour, and a timely stop at the Australian ANZAC memorial, where they were in time for the daily 11am ceremony and the Last Post. They also watched the Sydney fireworks display from Dawes Point in The Rocks, Sydney Aquarium, and Madame Tussaud's waxwork museum.

From Sydney, they flew down to Adelaide for the beginning of the 25th Australian Scout Jamboree, which had approximately 8500 scouts, 3500 adult leaders, and other staff at Tailem Bend. Rorie and five other New Zealanders were hosted by a troop from the ACT contingent, where he was a Patrol Leader. They spent time in their patrols doing the various activities that Jamboree had to offer, such as Wet & Windy, Trades, The Cube, Woodhouse Overnight, and The Metro Mania trip into Adelaide City. Alongside these activities, the scouts were rostered to Duty Patrol to collect and prepare food, cook, clean, and clear away the kitchen and cooking equipment.

Rorie administered his own treatment every 2nd day while away from home. This was facilitated by the NZ leaders while in Sydney, and by the medical staff and leaders while at the jamboree. His treatment was scheduled around the various activities he was involved in, as he was offsite for a significant part of each day. Apart from a couple of bleeding noses on the last 2 days (not surprising considering the heat of over 30 degrees most days), there were no incidents or injuries that required any further treatment.

Danny Webster: Leader

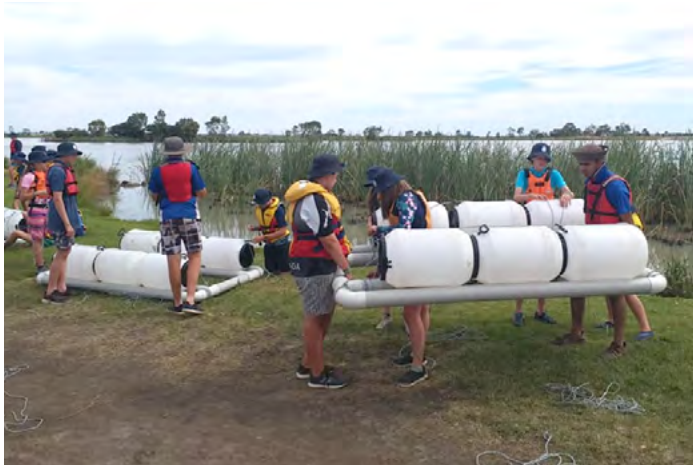
From a leader's perspective, I had met Rorie once prior to the jamboree and I was the line leader that accompanied Rorie and others in the host troop. Rorie was popular in the troop and other than the treatment requirements, he was treated as any other scout in the troop. I enjoyed getting to know Rorie and working with him throughout the jamboree and was encouraged by the way he didn't let his condition get the better of him or reduce the amount of fun that he had.

Mum and Dad

We are so proud of Rorie, going away for nearly three weeks on his own. A lot of planning and worry goes into a big trip like this. However, he will have memories for a lifetime. He met people from all over the world, saw amazing places, and did amazing things.

Kathy from Haemostasis was able to liaise with medical teams in Sydney and Adelaide and provide valuable contact numbers, let them know Rorie was coming to Australia, a Treatment Protocol and the necessary product and supplies. Rorie has always been well supported by Lincoln Scouts and huge gratitude and acknowledgement to the Leaders that went to Jamboree with Rorie, Scouts NZ, and Scouts Australia.

Haemophilia should not be a barrier for our young people.

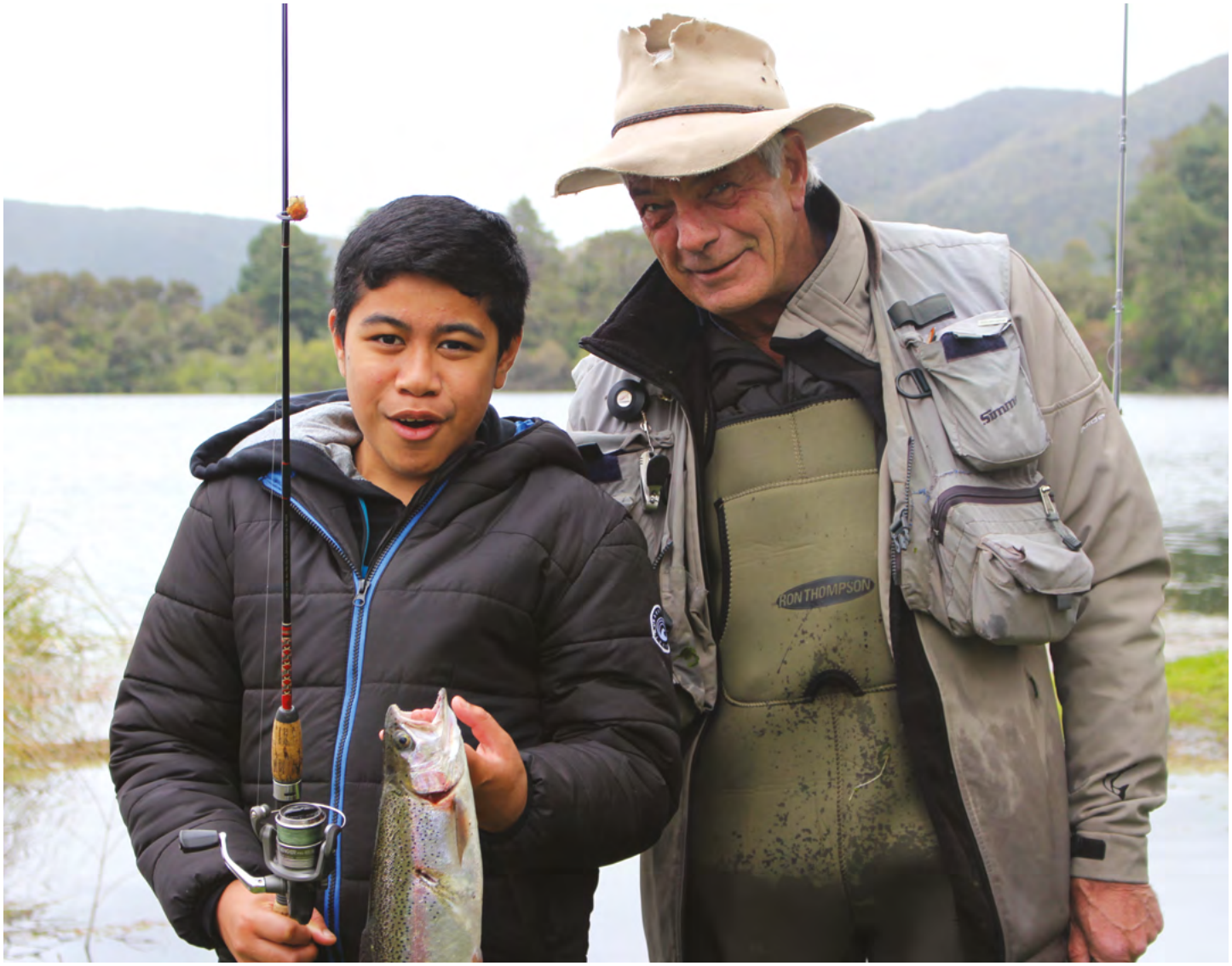


Images top to bottom: Rafting; Home base; Aceing the obstacle course

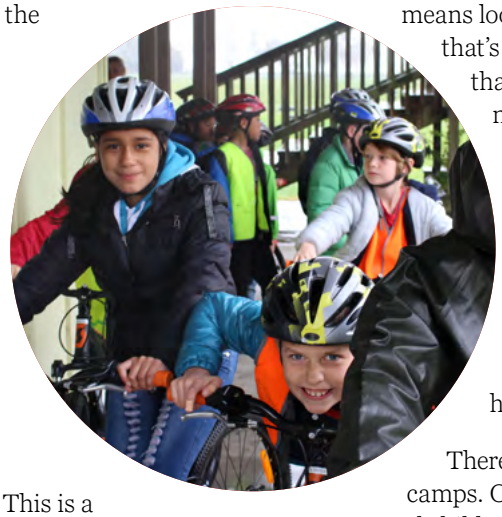
Coming Soon: National Family Camp

BY PHIL CONSTABLE

This month HFNZ are heading back to Camp Keswick in Rotorua for the National Family camp. This is a great opportunity for our young families to connect with others from around NZ, to participate in some fun social activities, and to learn new skills and information in the educational sessions.



Coming up this month, April 16 – 19, is the national Family Camp for families of children aged 0 – 10 with a diagnosed bleeding disorder. This event has been a long time coming. Originally slated for spring/early summer 2018, changes in staffing and organisation led us to reschedule the camp for early 2019, and then again, to the date we have now. While all the changes mean it's been a while since the last family camp, it also means we've had more time to plan and put together the programme.



This year we're heading back to our trusty Rotorua venue at Keswick Christian Camp. This is a familiar spot to many of you, as we've used it for several family camps in the past. We did give some thought to finding a fresh new venue, but by the time we'd decided on the dates it was too late to make a booking. We'll be looking for somewhere new to hold the next family camp instead.

The good thing about the Keswick camp venue is that we know it like the back of our hand. There are great facilities, plenty of space, fantastic access to the lake and surrounds, and they know us. That means we can run a really good camp, confident in the knowledge that we'll be able to keep it rolling no matter the weather.

Nicky Hollings, our former Northern, and current Midland Outreach Worker has taken the reins for this edition of family camp. She's been lucky to have a bit of mentoring from Colleen McKay, and the support of the HFNZ Outreach and admin staff. Nicky's experience with previous camps and workshops, along with her two trips to NACCHO, means that she's full of bright ideas about exciting things to do at camp.

With the HFNZ 60th anniversary event coming up later in the year, and it being an 18+ event, we wanted to make sure that our younger members had a chance to participate in the celebrations too. This led us to deciding on the theme of Back to the Future. For us, this means looking at and recognising all the hard work that's gone in over the last 60 years, and using that knowledge to inform what happens in the next 60. For our kids, that means knowing a bit about where the foundation came from and the battles and triumphs our people have had along the way.

The Back to the Future theme also lends itself to a bit of fun. We'll have appropriately named kids groups, and bring your 50s dress-ups, because we're having a sock-hop!

There are two key components to our family camps. One is making an opportunity for parents and children of families affected by a bleeding disorder to connect and support one another. The other is to supply the attendees with up-to-date information and education related to living with a bleeding disorder.

We have both of these bases covered at this camp. There will be many opportunities for adults and children to gather socially, in a less structured environment, so that they can get to know one another and share some of their experiences. We'll also have some top-notch speakers and educators along. These educational sessions will not necessarily relate directly to everyone who attends, however, we will be making sure that all attendees have access to content that is relevant to them.

Family camp is always a good time, even if the weather doesn't cooperate, and is one of the most valuable events HFNZ runs. We're really looking forward to getting going, and reconnecting with our whānau from around the country.

Registrations closed on April 1, but if you missed your chance, contact your Outreach Worker to see if there's still space.

Images top to bottom: Cycling; The 2016 campers; Raft building

World Haemophilia Day 2019: Outreach and identification

BY PHIL CONSTABLE

This year, the focus of World Haemophilia Day is reaching out and identifying new members of the bleeding disorders community. HFNZ have organised a number of venues around the country to light it up red to help us promote the day and the message.

17 April, 2019, marks the 29th World Haemophilia Day!

Last year, to celebrate we had a couple of landmarks in NZ light up red for the day. This year we've done much better, and we have up to 13 landmarks and buildings from around the country potentially lighting it up red just for us. Those confirmed include:

Christchurch:

- Fanfare sculpture
- Airport Arch
- Airport Control Tower and Terminal

Auckland:

- Town Hall
- Civic Theatre

Palmerston North:

- Clock tower

We also have positive indications, but no confirmation as of this writing from:

Dunedin:

- Municipal Chamber
- Railway Station
- Toitu Settlers Museum
- Wall St mall

Rotorua:

- Museum

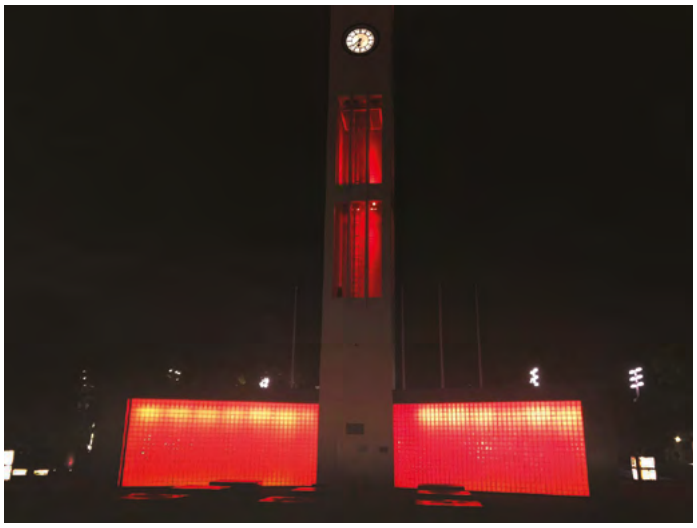
Hamilton:

Not yet specified

It's great to see three of our four main centres represented here. We appreciate the willingness of the local councils to get behind us and voluntarily support this initiative. It's amazing to feel so seen and valued by our local communities.

Of course, World Haemophilia Day (WHD) is an international initiative, led by the World Federation of Hemophilia (WFH). This year the WFH is promoting the theme: Outreach & Identification. Here's what they have to say about WHD 2019:

> *This year, the World Federation of Hemophilia (WFH) is focusing on reaching out and identifying new members of the bleeding disorders community. For many years, the WFH has supported outreach projects in different countries and conducted educational events for both lay and health care professionals in conjunction with our national member organizations (NMOs).*
>
> *The WHD2019 campaign will feature examples of WFH outreach initiatives such as organizing regional workshops, raising awareness of bleeding disorders through media coverage or training health professionals and collecting data. It will combine these examples with submissions from our community about outreach efforts in their own regions or ideas about what would be helpful for future initiatives.*
>
> *This landmark day also helps to illustrate the importance of the WFH Humanitarian Aid Program—an important endeavour for the WFH that provides a range of integrated care development training programs to ensure the local infrastructure and medical expertise are available to optimize and appropriately use donated products.*



If you're out and about on WHD this year, and you're in a town where one of these landmarks is lit up, please stop by and take a selfie. We'd love to see them, either to our email address: info@haemophilia.org.nz or via our FB page: https://www.facebook.com/haemophiliaNZ

Have a happy World Haemophilia Day!

Images top to bottom:
2018 Palmerston North clock tower;
Niagara Falls WHD 2017.
Image on left: Christchurch Airport - terminal

Global Haemophilia Advocacy Leadership Summit

BY SUE ELLIS AND DEON YORK

Organised by the Haemophilia Advocacy Advisors Board and Haemophilia Solutions by Bayer, the Global Haemophilia Advocacy Leadership Summit convenes global advocates to identify and discuss unmet needs in the haemophilia space. Held annually and now in its seventh year, the Summit provides a platform to discuss important issues affecting advocates and the training to implement change within their haemophilia communities. This year HFNZ president Deon York, and CEO Sue Ellis attended the Summit in Brussels.

On the 21st – 22nd of January, HFNZ CEO Sue Ellis and President Deon York attended the Seventh Annual Global Haemophilia Advocacy Leadership Summit in Brussels, Belgium. They were kindly sponsored by Bayer, including all travel and accommodation.

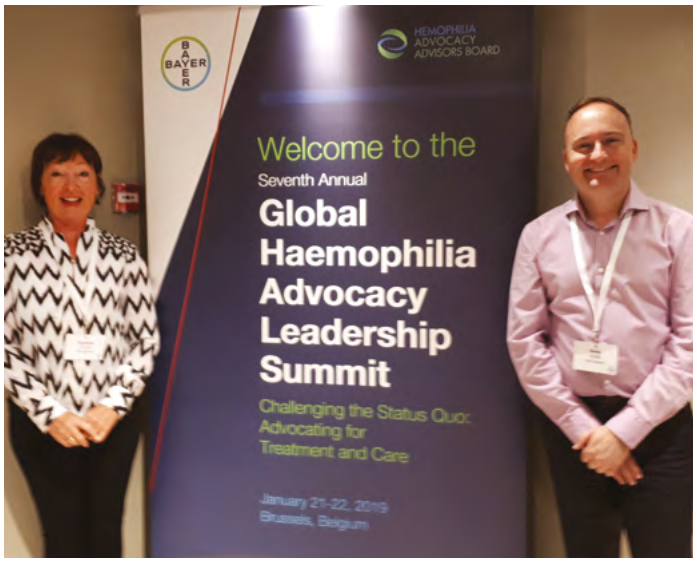
The purpose of these annual summits is to bring together advocates from around the world who are committed to improving the lives of those living with haemophilia - to build on lessons from previous summits; explore the important skills needed to be a leader in the haemophilia community; and to challenge the status quo and standard of access for patients. The summit was hosted by the Haemophilia Advocacy Advisory Board (HAAB), a group of leading patient advocates who were brought together to identify and address unmet advocacy needs.

The two-day programme included a mixture of presentations by leading international advocates, and workshops. The presentations looked at the issues of access-specific challenges that patients face, as well as the influence they have among stakeholders; regulatory processes and the evolving role of patients ensuring their voice is heard, including the patient-doctor dialogue; and innovative tools and resources available for evidence-based advocacy.

We heard from a number of internationally recognised leaders in the healthcare and rare disease space. Of particular interest was:

1. **Dr Diane Nugent**
   An expert in paediatric haematology with specific interests in blood/bleeding and clotting disorders, bone marrow failure and white cell and immune deficiencies. Diane presented an ACTION- Hemophilia Treatment Model, developed to establish specific treatment milestones, comprising seven milestones from sustaining life, minimal joint impairment, freedom from spontaneous bleeds, attainment of 'normal' mobility, able to sustain minor trauma to major surgery and culminating in a progressive definition of cure.

2. **Mark Skinner**
   A long time advocate and past President of the WFH, who introduced innovative access tools, based on research on real-world evidence and patient-reported outcomes. These included the Patient Reported Outcomes Burdens and Experiences (PROBE) project; the Cost of Haemophilia in Europe: Socioeconomic Survey (CHESS) study; the Haemophilia Experiences, Results and Opportunities (HERO) study and the WFH Annual Global survey data. Over the last few years, direct patient involvement in designing, conducting and evaluating research has emerged as a theme in Europe and North America to ensure research is centred on patients and their needs.



3. **Jamie O'Hara**
   A health economist presented on alternative payment models. The Pay-per-Patient model, a fixed payment model, based on utilization of a product for a defined patient, and the Pay-per-Outcome model which regards achievement of predefined outcomes. Jamie noted that bundled payment programmes, which encompass aspects of pay-per-patient models, have been effective in containing costs without compromising quality.

We also had fun working in groups including one to develop a role play to a set scenario. Our President, volunteered to lead our group to argue for better access to new therapies. It all felt very familiar!

We were also able to spend a little time outside the hotel to explore Brussels, albeit just the immediate surroundings. What we did get to see was awe inspiring, with the old-world architecture and cobbled streets covered in snow! We also had a delightful dinner as a group of approximately 70 attendees at a local restaurant – a great way to network and get to know other haemophilia leaders and advocates from around the world.

It was a valuable experience but also very tiring especially with four 12 hour flights and four one hour flights to travel the distance from the bottom of the world to Europe.
Thank you to Bayer for making it happen for Deon and Sue.

HFNZ 60th anniversary celebration

BY PHIL CONSTABLE

Plans are starting to come together for the HFNZ 60th anniversary celebrations in Wellington in November.

Here's what we're looking at so far:

On Friday November 8, we will have a welcome reception at Parliament. While the exact content and format of this is yet to be finalised, rest assured it will be a fun and appropriate opening for this very special weekend.

The main event of the weekend is the Gala Dinner on Saturday November 9. This will be held at Te Papa, and will include members, past and present staff, and special guests. Here you'll have the opportunity to mix and mingle with HFNZ people, past and present, and we promise we'll keep the speeches to a minimum.

As well as these two events, the youth committee is also looking at running an event on Saturday afternoon as part of our youth twinning with Nepal. Again, plans are afoot, but nothing has been finalised. Watch this space...

This is to be an 18 years and over event, and there will be a small fee attached to attendance. For those that don't require flights and accommodation the fee will be $50 per person, $100 per person for those who do.

Life members and special guests can expect an official invitation very soon. RSVPs will be due by June at the latest, so that we can organise flights, accommodation, catering, and seating.

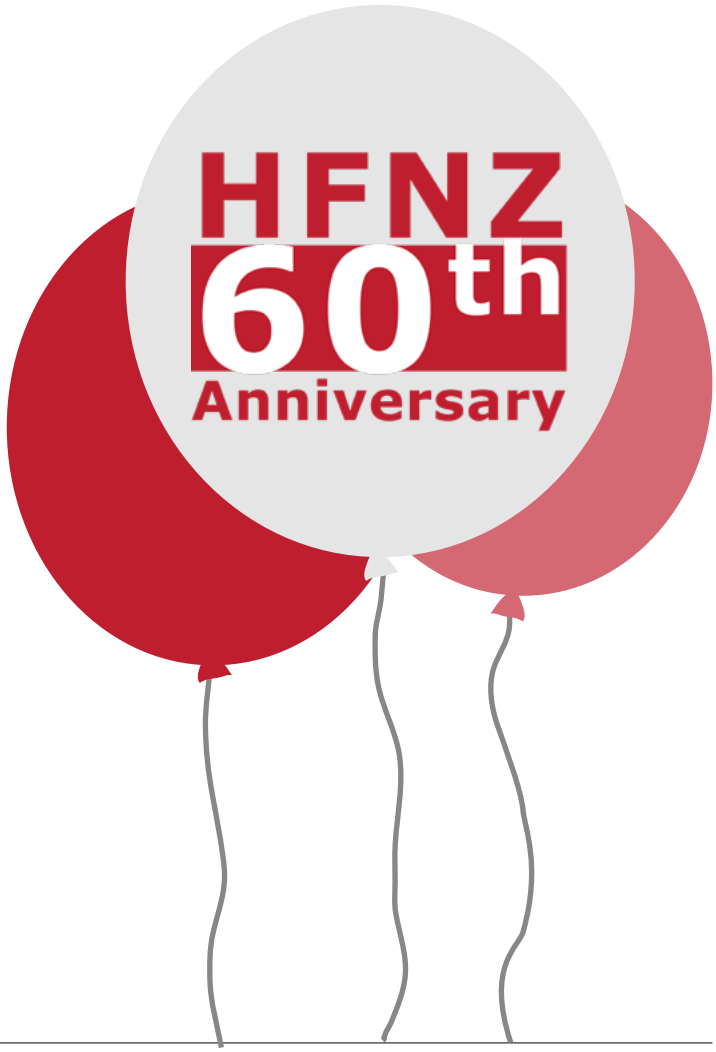
All other members and/or people connected to HFNZ are welcome to register today; you don't have to wait to receive your invitation. Just go to https://hfnz60.lilregie.com/ to register. For those who need flights and accommodation, we will contact you with options once you have registered to attend.

But remember, numbers are limited, so get in quick!

This is an important opportunity for us to celebrate the outstanding work done by members, staff, and supporters over the last 60 years. Without the courage and commitment of those men and women, we wouldn't be where we are today, with a strong voice, and world-leading comprehensive care.

It is also a chance for us to look to the future, to examine what the next 60 years might be like, for our people, and for HFNZ as a whole.

The HFNZ team looks forward to meeting and greeting you at this wonderful event.



Roche Emicizumab Advisory Board Meeting

BY SUE ELLIS

Patient Support Programme for People with Haemophilia A

On Saturday 2 February HFNZ President Deon York and I attended the inaugural Emicizumab Advisory Board meeting in Wellington. The meeting was initiated by Roche (Dr Christian De Ford), and facilitated by Atlantis Healthcare.

The objectives of the advisory board are:

- To understand the way Haemophilia Centres currently service and interact with their patients.
- To understand the potential impacts and increased support that the introduction of Hemlibra may require with regards to service and individual needs (practical and psychosocial) for people with Hemlibra and their families to optimise the overall Hemlibra experience.
- To co-create a solution to address the needs of both people with haemophilia A receiving Hemlibra and their HCPs to ensure the best possible outcomes for their treatment.

Health Psychologist, Dr Fiona Crichton, gave a presentation on the importance of taking into account the psychological aspects of treatment - or not - and the impact this can have on people's lives. I found this a very interesting presentation.

Each HTC was asked to outline the major issues they have for their centres and all noted parking and travel distance as the two main ones. Psychological support also featured, as did lack of enough physiotherapists. Waikato (Dr Julia-Anne Bell) noted the HFNZ camps were very helpful, gave parents confidence and the ability to advocate for themselves. Julie-Anne also noted how lucky they were to have Nicky in their region!

Palmerston North (Daryl Pollock) noted that they only have one Hematologist who works part-time 1 day/week, and no physiotherapist. They have money for one but this is for four hours per week.

Wellington was the only centre that mentioned HFNZ regarding the role of the Outreach Worker providing patient advocacy and education. BJ also gave a big plug for HFNZ camps.

Andrew Scott and John Tuck each spoke about their journeys with haemophilia, their past and ongoing issues with bleeding and joint damage, the psychological effects that had, and the huge impact and improvement using Hemlibra (emicizumab) has made to their lives – a game changer. Both speakers' stories were very moving and profound. It was wonderful to see how well Andrew looks.

The group of 18 participants including the two facilitators then divided into three groups to workshop what is required to support people with haemophilia and their families, the gaps and challenges, solutions, and priorities. Results from this workshop are to be written up and sent out to all attendees.

Notification

PHARMAC
TE PĀTAKA WHAIORANGA

Decision to make changes to funded haemophilia treatments

8 March 2019

What we're doing

We're pleased to announce changes to funded haemophilia treatments that will result in two new extended half-life treatments being funded for the first time in New Zealand. These extended half-life treatments will be funded for all people requiring prophylaxis treatment and are expected to provide prolonged protection from bleeding and reduce the number of intravenous injections required.

Changes are also being made to the funding arrangements of the currently funded short half-life treatments and bypassing agents.

The changes will be implemented over a six-month transition period, commencing on 1 May 2019.

These decisions arose from a Request for Proposals (RFP) process for the supply of haemophilia treatments. In addition to expanding the range of funded haemophilia treatments, this process will release significant funds for PHARMAC to invest in other pharmaceuticals for the benefit of New Zealanders.

Any changes to the original proposal?

This decision was subject to a consultation letter dated 29 January 2019.

There are no changes to the original proposal. We want to thank everyone who provided all their thoughtful feedback to this consultation, which has fed into this decision.

Who we think will be most interested

- Patients with haemophilia and their family, whānau or caregivers. The Haemophilia Foundation of New Zealand and any others who support people and families affected by haemophilia.
- Clinicians who treat patients with haemophilia, including the Haemophilia Treaters Group.
- The National Haemophilia Management Group, Haemophilia Treatment Centres, laboratories, the New Zealand Blood Service and suppliers.

Detail about this decision

New extended half-life treatments

From 1 May 2019, if considered appropriate by the treating clinician, all patients with haemophilia requiring prophylaxis treatment will have funded access to extended half-life Factor VIII and Factor IX treatments (Adynovate and Alprolix, respectively).

Other funding changes

- From 1 November 2019, Advate will be the Preferred Brand of short half-life recombinant Factor VIII, meaning that most patients with haemophilia A currently using Xyntha will need to change to Advate or Adynovate during a transition period commencing on 1 May 2019.
- From 1 November 2019, RIXUBIS will have Sole Subsidised Supply status for short half-life recombinant Factor IX, meaning that all patients with haemophilia B currently using BeneFIX will need to change to RIXUBIS or Alprolix during a transition period commencing on 1 May 2019.
- If a person is clinically unable to transition between brands of Factor VIII from Xyntha to Advate or Adynovate, an application will be required from the treating clinician to the Haemophilia Treaters Group prior to 1 November 2019 for the patient to remain on Xyntha. Patients will need to fulfil one or more of the following eligibility criteria set by PHARMAC for funded access to Xyntha:
  - previously had high titre inhibitor levels
  - are undergoing active or have undergone immune tolerance therapy
  - have a known product allergy
  - have recently commenced therapy (Previously Untreated Patients or PUPs)
  - live in the same residential setting with other people with haemophilia who are unable to switch, as it would be safer to have only one brand kept in the household.
- Existing approvals for Kogenate FS will remain valid.
- From 1 November 2019, FEIBA NF will be the Preferred Brand of bypassing agent for patients requiring greater than 14 days predicted use.

Summary

The table below provides a summary of the funding arrangements that will apply from 1 November 2019, following the six-month transition period:

rFVIII	Short half-life	**Preferred Brand** (unrestricted access)	Advate (Shire)
		Rare Clinical Circumstances Brands (access determined by Haemophilia Treaters Group on a named patient basis)	Xyntha (Pfizer) Kogenate FS (Bayer)
	Extended half-life	**Sole Subsidised Supply** (unrestricted access for prophylaxis only)	Adynovate (Shire)
rFIX	Short half-life	**Sole Subsidised Supply** (unrestricted access)	RIXUBIS (Shire)
	Extended half-life	**Sole Subsidised Supply** (unrestricted access for prophylaxis only)	Alprolix (Sanofi/Bioverativ)
Bypassing agents (FEIBA and rFVIIa)	<14 days predicted use	**Multiple Supply** (unrestricted access)	FEIBA NF (Shire) NovoSeven RT (Novo Nordisk)
	>14 days predicted use	**Preferred Brand** (unrestricted access)	FEIBA NF (Shire)
		Rare Clinical Circumstances Brand (access determined by Haemophilia Treaters Group on a named patient basis)	NovoSeven RT (Novo Nordisk)

Please see the consultation letter for additional detail.

Our response to what you told us

We're really grateful for the time people took to respond to this consultation. A summary of the main themes raised in feedback and our responses is shown below.

Theme	PHARMAC response
Responders were generally supportive of the proposal to fund extended half-life Factor VIII and Factor IX. Responders noted that whilst some patients will find the change unsettling, it is likely that most (particularly those with severe haemophilia A and B) will want to change to extended half-life Factor VIII and Factor IX, particularly if the right patient education is in place.	This is consistent with our view on likely uptake of extended half-life treatments. We will focus our implementation activities on supporting people with haemophilia to change to the different treatments, ensuring Treaters have sufficient information on the changes and the new products to be able to support their patients to change treatments. We will work with the Haemophilia Foundation to support their members during the transition period.
Choice of particular products/brands	
There are differences in the half-lives of the extended half-life Factor VIII treatments available and this should be considered in the evaluation.	At its meeting on 4 October 2017, the Haematology Subcommittee advised that PEG-rFVIII (Adynovate) could be considered to be clinically equivalent to rFVIIIFc (Eloctate) for the purposes of funding an extended half-life Factor VIII. This was based on its view of the available evidence that both PEG-rFVIII and rFVIIIFc have the same or similar therapeutic efficacy in both adults and children, with the same or similar risks. This advice was considered in the RFP process.
There is limited data on long term safety of regular PEG-rFVIII (Adynovate), particularly in infants and children.	Adynovate is registered by Medsafe for use in children in New Zealand and in many other overseas jurisdictions including Australia, Canada and the United States. However, there would be no requirement to treat children with Adynovate. The short-acting product, Advate, will be an available funded alternative if treaters or patients consider that Adynovate is not appropriate for them. We acknowledge that there is a lack of long-term data confirming the safety of this and many other pharmaceuticals used in children. The Haematology Subcommittee noted, in its consideration of Adynovate, that there were no polyethylene glycol (PEG) related adverse reactions in the clinical trials. We note that in Canada, Adynovate will be the only brand of extended half-life Factor VIII funded. The Canadian Blood Services outlined the widespread and long-term use of PEG containing pharmaceuticals during implementation of that decision, noting that no safety concerns have been raised. We will also address this topic in more detail during our implementation activities.

Other out-of-scope haemophilia treatments	
Emicizumab could radically change how haemophilia A is treated, both for patients with and without inhibitors.	Novel agents such as the as subcutaneously administered prophylaxis treatment emicizumab were specifically excluded from the RFP. This was primarily for the reason that, at the time, we had not yet received clinical advice or performed our assessment on any of these products. Any products excluded from the scope of the RFP could be listed at any time during the RFP period, but would require funding application assessment and ranking against all of PHARMACs other options for investment. Since the release of the RFP a funding application for emicizumab in people with haemophilia A and inhibitors to FVIII has been received and was considered at the recent meeting of the Haematology Subcommittee in January 2019. Minutes of this meeting are not yet available, but will be published on the PHARMAC website once finalised.

If you have any questions about this decision, you can email us at enquiry@pharmac.govt.nz; or call our toll free number (9 am to 5 pm, Monday to Friday) on 0800 66 00 50.

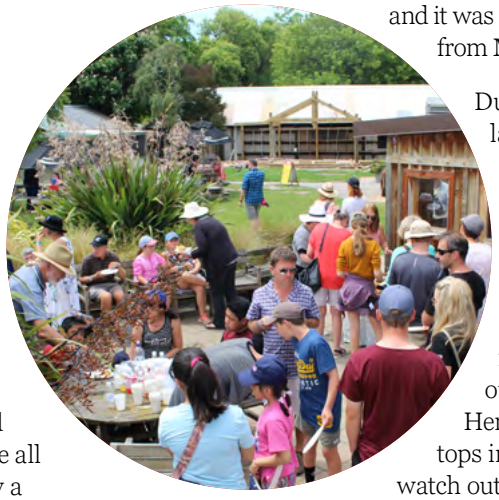
Region group reports

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 since their last reports, and what they have coming up.

CENTRAL REPORT

By Stephanie Coulman

Central region ended 2018 with a visit to Southward Car Museum in late November. This was a really well attended event. We had a festive lunch followed by a tour of the Museum's collection of cars. Our Men's fishing trip was to have been held on 10 March 2019. We had filled the 20 spaces on the boat with men from across the region. Unfortunately, this outing had to be postponed due to the terrible weather on the day. The Central team have rebooked the trip for June 15. It was a shame for those all set and ready to catch the big one, and enjoy a morning on the water.



Looking ahead to 2019, we have the following events planned:
17 April: World Haemophilia Day dinners in Wellington & Palmerston North. To tie in with the youth twinning project with Nepal, we'll be holding these dinners at a Nepalese restaurant. Invites will be sent out shortly.

August: Central AGM in Wellington, at Carter Observatory.
8 - 10 November HFNZ 60th anniversary celebrations in Wellington. 2019 is an exciting year for us with the Foundation's 60th celebrations being held in our region. We encourage as many of our members as possible to attend this event.

MIDLAND REPORT

By Tineke Maoate

Hi everyone.
The Midland region is happy to be hosting the National Family Camp in Rotorua again this year. We're looking forward to all the families that can manage it coming to participate and support each other. These camps are very important for our new families to get educated and to meet other families. We're excited to have a great camp put together by our wonderful our Outreach Workers. The committee is working together to organise other events for the year ahead. Please remember our 60th celebrations in November. Look after yourselves and each other.

NORTHERN REPORT

By Greg Jamieson

The Northern region linked up with the Midlands region and held our Christmas event at Rainbow's End. This was a great day out and it was nice to spend some time with families from Midland.

Due to the low number of RSVPs for camp last year, we decided to hold a summer get together at the Grounds in Henderson. This was a lovely way to spend some time together in a relaxed environment. Amber, our new Outreach Worker, attended. She made time for everyone and connected well with the Northern members. She will be a good addition to our team so reach out and say hello.
Hemi is arranging our next event at the tree tops in Woodhill in the first week of May so watch out for this.

We welcome Rosalie Glynn to our Northern committee. It's nice to have some new ideas and her experience as part of our team. If anyone is interested in joining the committee, please get in touch with Amber. The committee arranges events for the Northern region and you can contribute as much or as little as you are able to.
Lastly, there seems to have been an issue with some of our members not receiving invites. If you didn't receive invites to the Grounds or to Rainbow's End, please let Amber know so we can get this fixed.

SOUTHERN REPORT

By Zac Porter

Tēnā koutou,

The Southern region has been busy ticking away recently. We thank the Waimakariri Jet Boating Association for hosting some of our members for a day out on the river. It sounds like this family day was great fun.

We have our annual fundraising play coming up on the 25th of May at the Elmwood school theatre. Tickets will be $22 each and it would be great to see all our Southern members there. If you would like a ticket, please get in touch with Zac at zac_porter@hotmail.com. Also, if you can donate any food or raffle prizes we would love to hear from you.

We are also looking at hosting a Southern Youth event to Scared Scriptless, so keep an eye out for your invite soon if you are a youth member.

News from around the world

RECENT DEVELOPMENTS IN HEMOPHILIA: GENE THERAPY

By Mike Bassett

Gene therapy for hemophilia is starting to come of age and promises to transform the way patients with this rare blood disease are treated.

"The concept of gene therapy has sort of been at the root of where we've been heading in the treatment of hemophilia from the very beginning," Steven Pipe, MD, of the University of Michigan in Ann Arbor, told MedPage Today.

Usually an inherited disorder, hemophilia patients have a defective gene that causes them to lack proteins necessary for blood clotting -- factor VIII in the case of patients with hemophilia A, or factor IX in the case of hemophilia B, Pipe pointed out.

"In the recombinant DNA era, we've been taking a good copy of the factor VIII and IX gene and putting it in cells, and making recombinant copies of factor VIII or IX replacement protein that patients have been infusing," he said. "That kind of replacement therapy has been the foundation for how we've treated hemophilia now for several decades."

With gene therapy, the concept remains the same, Pipe explained: "If we can get a good copy of the factor VIII or IX gene to the patients' own cells, then they could make their own factor VIII or IX and also maintain steady-state levels. And that really has been an aspirational goal -- to see this happen in hemophilia."

From a safety and efficacy standpoint, the gene therapy platform that is clearly the most promising is the adeno-associated viral (AAV) vector.

In a landmark study presented at the 2011 American Society of Hematology (ASH) meeting and published in the New England Journal of Medicine, Nathwani and colleagues reported that a one-time infusion of AAV vector-expressing factor IX led to sustained increases in factor IX production in six patients with severe hemophilia B.

This approach "gives us an opportunity to do the gene delivery in a targeted fashion to the liver," said Pipe. "And the clinical trial results in the past 10 years have shown we can do this safely, and we can achieve [factor activity] levels that are transformational for patients from a phenotype perspective."

What became clear, Pipe added, was that the success of gene therapy would come out of a partnership between bioengineered molecules and a gene therapy delivery platform such as AAV.

Pipe is the principle investigator of uniQure's phase III HOPE-B pivotal study of AMT-061, an investigational AAV-based gene therapy incorporating the factor IX-Padua variant for the treatment of patients with severe and moderately severe hemophilia B.

The company's ongoing phase I/II trial of its first-generation AAV-based gene therapy, AMT-160, consists of an AAV5 vector carrying gene cassette with the standard wild-type factor IX gene.

While that earlier trial has been successful, Pipe noted that by substituting the bioengineered Padua variant -- which generates a hyperactive form of factor IX -- for the wild-type factor IX gene, investigators have been able to achieve factor IX levels of 25% to 50% of normal with AMT-061, compared with levels of 5% to 10% with AMT-060.

Another gene therapy for hemophilia B, SPK-9001, is being developed by Spark Therapeutics and Pfizer and is based on the Padua variant. The therapy has proven to be effective in reducing bleeding and factor IX infusions in phase I/II and is now in phase III testing.

"We had sustained expression of factor IX -- an average of 35% for 15 hemophilia B patients," said principal investigator Lindsey George, MD, of Children's Hospital of Philadelphia. "From a safety standpoint there were no major safety concerns, and no adverse events."

"We know with hemophilia that the clinical phenotype is closely correlated with what your factor activity is, so with mean factor levels of 35% we weren't surprised to see a dramatic alteration of the phenotype in patients," she told MedPage Today, noting that there was almost a complete resolution of bleeding, with one patient having a bleeding event that was remarkably reduced from his baseline.

"And then along those same lines there has been virtual elimination of the requirement of factor use, which is the traditional standard of care for hemophilia," George said. "So, the efficacy has been particularly striking."

"This partnership between these bioengineered molecules and the AAV platform is proving to be very successful," said Pipe. "And we are seeing the same thing happen with factor VIII."

George is also the principle investigator for Spark's SPK-8011 phase I/II trial for hemophilia A, for which preliminary data was reported at the 2018 ASH meeting.

She noted that no safety concerns have emerged in the trial and that from the preliminary efficacy data, even the expression of a

modest amount of factor results is a "really impressive improvement in the clinical phenotype," similar to what has been seen in the hemophilia B study.

With these advances in gene therapy, is it too early to talk about a cure for hemophilia?

"It always makes me nervous to say 'clinical cure,'" said George, who pointed out that investigators still don't have information about the duration of expression in patients treated with these therapies. But she added that the men from the first successful hemophilia B trial have had stable expression for about 10 years, and this duration of expression has also been observed in large animal models.

"It's reasonable to predict you would have some sustained expression from the therapy throughout your adult lifetime, but guaranteeing your factor level would be X percent throughout your lifetime is where the question mark is," she said.

However, she noted that the men in that hemophilia trial haven't had bleeding events and haven't had to use factor. "From a clinical standpoint they really haven't had any manifestations of their hemophilia, so I guess you could characterize that as a phenotypic cure," she said. "But, I think we need more time before we can say that with complete confidence."

Pipe also noted that researchers are working on different iterations of AAV, "which will give us some needed choice related to the eligibility of patients."

tEligibility for gene therapy is a potential barrier for hemophilia patients because AAVs are viruses that can produce an immune response in patients -- perhaps in as many as half of patients.

"With some additional engineering some companies have been able to reduce the screen failures for clinical trials -- getting it downwards to 25% to 30%," said Pipe. "That's still a disappointing impairment for enrollment in clinical trials. That's why we are excited to see multiple capsid platforms being developed in the research community, because we anticipate we are going to need multiple versions of these AAV vectors to maximize the eligibility for patients."

As for the eventual commercialization of a gene therapy product, George predicted there is likely to be a licensed product available within 2 to 5 years. "My guess is there will be quite a few licensed products for hemophilia A and B that over time will really have the potential to alter the paradigm of how we treat hemophilia patients," she said.

Source: https://www.medpagetoday.com/recent-developments/hemophilia/78500

CONGENITAL BLEEDING DISORDERS NOT ASSOCIATED WITH INCREASED ENDOSCOPIC BLEEDING RISK WITH PROPER PROPHYLAXIS

By Megan Garlapow, PhD

Medical interventions may pose an extra bleeding risk for patients with congenital bleeding disorders, but the level of risk is not well understood for all procedures or in the context of prophylactic therapies. Researchers examined bleeding risk during gastrointestinal endoscopic procedures in this patient population and presented results in a recent issue of Haemophilia.

Patients examined in this retrospective chart review conducted at McGill University Health Centre in Canada were adults (48 patients) with hemophilia A or B, factor VII deficiency, factor XI deficiency, or von Willebrand disease who had undergone at least 1 gastrointestinal endoscopy (104 endoscopies overall). Patients received periprocedure bleeding prophylaxis therapies as appropriate. The primary study outcome was the bleeding rate at 72 hours after endoscopy.

The bleeding rate among patients in this study during the 72 hours after endoscopy was 0.96% (95% confidence interval [CI]: 0.17%-5.25%). For hemophilia A, the most commonly represented condition in this study, the rate was 2.2% (95% CI: 0.4%-11.3%).

According to the authors, the 72-hour bleeding rate within the general population following high-risk endoscopies is 0.3% to 10%. Of note, one type of high-risk endoscopy examined in this study, colonoscopy with polypectomy, had a 72-hour bleeding rate of 4.8% (95% CI: 0.9%-22.7%), or 1 out of 21 patients with congenital bleeding disorders. However, this falls within the range of bleeding with high-risk endoscopies reported for the general population. The bleeding rate for all other bleeding disorders was 0%.

In this study population, endoscopy did not significantly increase bleeding risk in patients with congenital bleeding disorders who were receiving proper management. The authors urged further analysis with larger studies to fully ascertain this risk and the effectiveness of prophylaxis therapies.

Reference

1. Tomaszewski M, Bienz M, Kherad O, et al. Low endoscopy bleeding risk in patients with congenital bleeding disorders [published online February 12, 2019]. Haemophilia. doi: 10.1111/hae.13691

Source: https://www.hematologyadvisor.com/home/topics/bleeding-disorders/congenital-bleeding-disorders-not-associated-increased-endoscopic-bleeding-risk-proper-prophylaxis/

NFC volunteers

THE YEAR AHEAD

April 16 - 19, 2019

- National Family Camp
  Keswick Christian Camp, Rotorua

April 17, 2019

- World Haemophilia Day
  Multiple venues around NZ

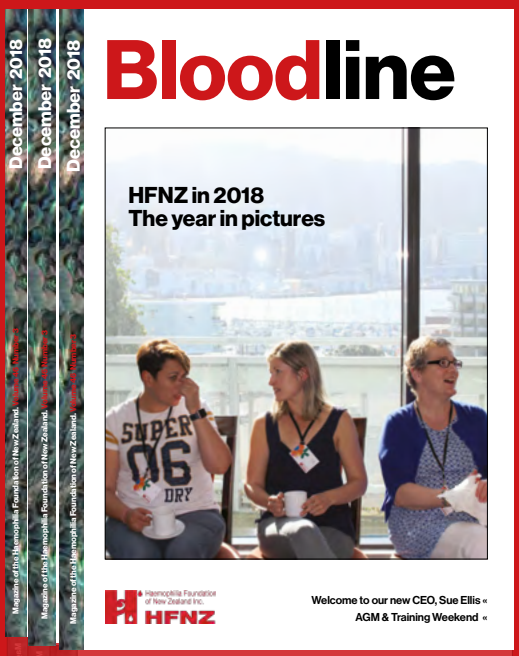
October 19 & 20, 2019

- HFNZ AGM and Regional Office-holder Workshops
  Rotorua

November 8 - 10, 2019

- HFNZ 60th Anniversary Celebrations
  Wellington

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



Enjoy this issue ONLINE

Check your inbox for the online version today.

Reading Bloodline online saves HFNZ money, and is good for the environment.

Outreach Freephone
0508 322 867
www.haemophilia.org.nz