Bloodline

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Bloodline

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CONTENTS

Disclaimer: The information contained in this magazine is not intended to take the place of medical advice from your GP, haematologist, or specialist. Opinions expressed are not necessarily those of HFNZ.

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

All too often we are so focused on the plans ahead that we don't take the time to look back, reflect on our achievements, and celebrate how far we have come. Our 60th anniversary celebrations were the time to do just that.

Winding back and looking forward

When the founders of the Haemophilia Society first gathered in a dusty town hall in Wellington back in 1958, could they have imagined how the society that is now the Haemophilia Foundation of New Zealand (HFNZ) would have transformed 60 years later? Your lives, your stories, and your achievements all form part of what HFNZ is today.

Our 60th anniversary celebrations in early November offered a rare opportunity to recognise our achievements and milestones together, reminisce, connect, and reconnect. From the welcome reception at Parliament to the gala dinner at our national museum, Te Papa Tongarewa, these celebrations are now another chapter in our history.

Our look back on 60 years delivered a number of firsts for us: the first time a sitting president of the World Federation of Hemophilia attended an HFNZ event in New Zealand; the first time a sitting member of cabinet welcomed us at Parliament, the first time we welcomed guests from Nepal (our youth 'twin'), and the first time Piritoto showcased to us all how they enrich our community, to name but a few.

Now we have looked back, it's also important to continue to look forward. The feedback on the weekend's events has been overwhelmingly positive and we are in a stronger position to tackle the future with a renewed passion for everyone who makes up our eclectic and inspiring community. In reflecting on our past, we can only be inspired to look forward to a promising future together.

Thank you to all of you who were able to be part of our 60th anniversary celebrations.

Switching around and switching up

This year the majority of people living with haemophilia A or B have now switched to longer-acting FVIII or FIX. Change can be daunting. When I opened my new product, it struck me how much paraphernalia there was, and it all felt slightly old school. Ultimately, it is not the packaging or even the administration kit that matters, it's what's inside that counts. For haemophilia A the longer-acting product is still an improvement on standard half-life (i.e. how long it stays in your system). For haemophilia B, the longer-acting product is verging on transformative. My take-home message is: although the new therapies may appear like a nod to the past (if you remember the late nineties), it's what's on the inside that counts. As for novel therapies, HFNZ still follows developments with interest.

Scanning the horizon

What does 2020 hold for HFNZ? In this issue you will see a calendar of events planned for 2020, so lock and load your diaries. What are our aspirations for our members? We cannot answer this on our own, so we will conduct another member survey in the first half of 2020. We want your feedback so we can deliver what matters to you. I can offer you my hopes for 2020:

- we do not lose sight of the importance of comprehensive care for the treatment of haemophilia and related bleeding disorders;
- we investigate and respectfully challenge the well-established assumptions about what 'adequate' means in terms of care;
- we remain informed about the explosion of therapies available and emerging. For instance, how ready is New Zealand to consider funding gene therapy if it became available tomorrow? and
- we remember that pain management is still an important issue in our community, and some are still limited by their ailments, leading to limited access to education and employment.

Above all, we must foster kindness in our community. There are still psychosocial issues that hold people back from realising their full potential. If these cannot be openly discussed among our community, where can they be discussed?

I hope that you all had some much-needed rest and relaxation over summer, and may the new year bring us all 20/20 vision.

Deon York

HFNZ President

From the CEO

BY SUE ELLIS

I recently attended the 11TH WFH Global Forum in Montreal. As the snow fell outside, I sat down to listen to the presentations from experts on research and treatment products for bleeding disorders and reflected on learnings from the past year since I started in the CEO role on 14th November 2018.

- I now know a lot more about haemophilia and bleeding disorders and the various abbreviated titles!
- I have learnt that it's not just about haemophilia but the Foundation encompasses all people with bleeding disorders and their families.
- That we are a family of members and staff who collectively are dedicated and passionate about advocating for access to the very best treatment and care.
- Attendances to international forums such as the WFH Global Forum, the Global Haemophilia Advocacy Leadership Summit and the recent Haemophilia Foundation of Australia conference and listening to experts in the bleeding disorder world, tells me we are moving quickly towards new and exciting treatment products and gene therapy. Gene therapy will not only change the life of people with bleeding disorders, but also a paradigm shift in haemophilia care (but costs will need to come down!).
- Although we are a small country, we are holding our own in terms of access to affordable treatments (always to be improved of course!) and ongoing care.
- However, we are not smug about this, and continue working to help less advantaged countries through the WFH Twinning programme – 70 to 75% of people in developing countries remain undiagnosed.
- Funding via Kiwifirst donations and support from our pharma friends and bequests tells me how valued we are to the bleeding disorders community.

The WFH Global Forum

As a brief summary of the WFH Forum, the presentations were focused on safety, supply, and access to treatment products. Speakers presented on pathogen safety, emerging risks, and the WHO efforts to advance quality and availability of blood components, and extended half-life products. There were also presentations on point-of-care diagnosis, including FVIII monitoring devices, and ensuring access to affordable treatment products and humanitarian aid. Brian O'Mahony, CEO of the Ireland Haemophilia Society, spoke on tenders and procurement strategies for access to treatment, which obviously works, as Ireland now has funded Emicizumab, not only for patients with inhibitors but also non-inhibitors.

There was also a focus on research and haemophilia treatment, including new and novel haemophilia therapies, with positive commentary on the future of gene therapy treatments. I was saddened to hear of the lack of women in clinical trials, the number of reported women was still too low and still infrequently included in national databases. Indeed, I noted that not only were presentations about women and bleeding disorders for women left to the end of the last session on the last day, half the audience had left.

The 60th Anniversary

Our 60th anniversary celebrations were such a fabulous success and I really enjoyed meeting many members (where I was frequently introduced as the "new" CEO!). All thanks to the planning team that ensured all the details were ticked off. I want to reiterate this would not have been the success it was without the vision, passion, belief, and commitment of our President Deon York.

I hope everyone had a great Christmas break. Welcome to the new decade and new beginnings.

HNFZ 60th Anniversary

HFNZ finished the year with the 60th anniversary celebrations in Wellington. We celebrated our diamond anniversary with a welcome reception at parliament, and a gala dinner at Te Papa Tongarewa. For those of you unable to attend, here's an overview of what went on, the speeches from the welcome reception, and some fantastic images of the weekend.

The biggest social occasion of the HFNZ year was our 60th anniversary celebration in Wellington. Over the weekend of November 8 – 10 HFNZ members from around the country, and special guests from around the world, gathered to acknowledge our foundation's journey from the first meeting in a hall in Lower Hutt in 1958.

The weekend included two main events, and a number of other gatherings of representative groups from within HFNZ.

The first big event was the formal welcome, held in the Grand Hall at parliament, and hosted by government minister, Hon Chris Hipkins. This was the formal part of the weekend, where nearly 200 members and guests had the opportunity to renew acquaintances, before being treated to a few words from some of our special people.

HFNZ president Deon York got things underway with a journey by the numbers through the history of HFNZ. He then introduced Chris Hipkins, who welcomed us to our parliament, and outlined his own connection to the foundation. This was followed by a few words from HFNZ patron Elizabeth Berry, former president Mike Carnahan, and clinician Claire McLintock, before CEO Sue Ellis concluded the speeches with some appreciations and acknowledgements.

You can read some of the speeches later in this issue of Bloodline.

Following the formal part of the night, people continued to mix and mingle. They enjoyed the opportunity to reconnect and socialise in this wonderful setting.

The second big event was the gala dinner at Te Papa Tongarewa, the Museum of New Zealand. Again, we had close to 200 members and guests attend this fabulous event. The evening started with music and socialising in the lobby, where guests enjoyed a drink and canapés. We then moved up the stairs to the dining area, where Piritoto performed waiata from different regions of Aotearoa to welcome the guests. This was very well received, and very professionally performed.

Dinner service followed. The courses, washed down with two specially branded HFNZ wines, were punctuated by some speeches and presentations. Notably, Kyle Cunningham was awarded the Elizabeth Berry Exercise Cup by the patron herself, Piritoto were presented with a beautiful tokotoko to recognise their 10th anniversary, WFH president Alain Weill said a few words, and Lauren Phillips spoke about the activities of the National Youth Committee, and introduced us to the youth visitors from Nepal.

It was lovely to see members and guests enjoying themselves, and honouring all the work and commitment that has gone into the last 60 years. The gala dinner was a huge success.

The birthday cake. 60 years, Bloody Marvellous

In addition, other events occurred alongside the 60th celebrations.

The National Youth Committee held a three-day leadership-training workshop, where they continued the development of the next cohort of HFNZ leaders. This event was notable because they had four young leaders from Nepal in attendance. This was part of the ongoing WFH twinning programme with which the NYC is involved. It was wonderful to see these young people experiencing a bit of Kiwi culture, and expanding their horizons.

There were also smaller gatherings. Members with von Willebrands and platelet disorders rode on the cable car and had morning tea at the top, a small group of Masters men enjoyed morning tea and a chat at the Bay Plaza, and current and former HFNZ staff got together for breakfast.

All in all this was a fantastic weekend, which appropriately celebrated the amazing milestone of 60 years of the Haemophilia Foundation of New Zealand. The foundation acknowledges the support of our enduring partners Kiwifirst, and sponsors Takeda, Roche, Sanofi, and NZ Blood, as well as the work of the organising committee in making this celebration possible.

Te Papa Tongarewa was a fantastic venue

WFH president Alain Weill addresses members and guests

Kyle Cunningham accepts the Elizabeth Berry exercise cup from the patron

Lauren Phillips and Hemi Waretini with the Nepalese youth delegates.

HFNZ CEO Sue Ellis

Haematologist Claire McLintock

Hon Chris Hipkins welcomes HFNZ members and guests to parliament

WFH President Alain Weill, Deon York, and the Nepalese Youth delegation

Piritoto perform kapa haka

Deon York presents Piritoto chair Tuatahi Nightingale-Pene with a tokotoko to recognise their tenth anniversary

The Piritoto representatives with their tenth anniversary taonga

Members and guests danced into the night

Jade Paul, Claire McLintock, Chris Hipkins, and Deon York

The Youth team joined us from their weekend leadership workshop

Let the dancing begin...

Lauren and Hemi talk twinning at the gala dinner

Deon York: President, HFNZ

Friday 8 November, 2019. Grand Hall, Parliament.

60 years of the Haemophilia Foundation of NZ, formerly the Haemophilia Society. Once again, I would like to add my welcome to you all.

It's our diamond anniversary. Like the formation of a diamond, as we continue our work for all people in New Zealand affected by inherited bleeding disorders, there will pressure, it will get heated, and it will take time, but the end-result will be worth it – it's worth it when you see the results.

If I were to choose between numbers or words, I would say I'm more of a words person. Numbers can also tell a story. So, let's reveal some of those numbers that take us through but a mere snapshot of the past 60 years of the Haemophilia Foundation of New Zealand.

12%: The estimated number of people with haemophilia who survived adolescence in the 1950s. In today's context that's quite a staggering figure.

21 February 1957: The date that a letter appeared in the Evening Post, by Bob White, a man with haemophilia, recent immigrant from the UK, seeing who else was out there. He was to become the founder and first honorary chairman. Bill Carnahan, Mike's father, became the first honorary secretary. Bob was described by Mike: 'A clunky, disabled guy came around to speak to my father. He had a loud voice, had his legs in callipers, and grumbled about the government. All this seemed to suit my father, and so they decided to form a society'. Auspicious beginnings.¬

13 May 1958: You should all know this one. This is the year we were founded. For the astute among you, you will realise that we are in fact older than 60 years. So tonight, and over this weekend, we celebrate the first 60 years of HFNZ. This was the date of our first general meeting, in Lower Hutt.

64: The number of listed members in 1958. Of note, both clinicians and people with bleeding disorders. This partnership, rare at the time, and still rare today.

1,400: The approximate number of members of the Haemophilia Foundation of NZ in 2018. – 60 years on.

350 pounds: The amount raised in 1963 for Roy Jones so he could travel to work from Stokes Valley to Trentham in Wellington. Fully paid with interest by 1966. They certainly had a tighter rein on the membership back in 1963.

1: John Davy bought a huge bag of peanut flour from South Africa as the thinking at the time was that eating copious amounts of peanuts improved clotting. Among some members you should never mention 'peanut brownie.

1971: HFNZ became an associate member of the WFH. We became a full member in 1975.

$1.15: The value of the only fixed asset we had in 1971, a stapler. What high rollers we are.

1976: First family camp

1977: Mrs Maryke Bergers – first field officer appointed. It was also the same year that there were discussions about a haemophilia register. We have had an expansion of staff since then, but we are still talking about the accuracy of the register. Steph 1989.

1979: ACEET

29: The number of people with haemophilia who contracted HIV through blood in the 80s

188: The number of people with haemophilia contracted hepatitis C through contaminated blood products in the 90s.

$20: 29 July 1996 to be exact. The first $20 donated through Kiwifirst from a donor in Naenae here in Wellington. Today the partnership with Kiwifirst continues, and we would be nowhere near where we are without the generous donations of the NZ public. Our staff and our programmes would be but a fraction of what they are today.

1998: Recombinant and prophylaxis

2003: The first national women's workshop weekend was held at Blue Skies, Kaiapoi. Also the year our first CEO joined. Coincidence? Well, Colleen had joined 5 years prior as well.

30 November 2004: The unveiling of the HFNZ rock in the circle of friends memorial garden at Western Springs in Auckland (Tony Goodwin)

2006: The year a treatment and welfare package was delivered for all people who contracted HCV through contaminated blood products.

2010: The first New Zealander was elected to the board of the World Federation of Hemophilia. I have absolutely no idea who that was.

80: The number of members who attended the WFH World Congress in 2014 in Melbourne, the largest contingent and a mammoth fundraising effort.

9: The number of presidents HFNZ has had. We've also had many vice presidents, treasurers, secretaries, regional delegates, local committee members. We've seen it all I can tell you.

10: The number of years since piri toto was founded.

35: The approximate number of national events run over the past 10 years. There has been a concerted effort to run programmes targeted at all parts of our community. As treatment has improved for people living with severe haemophilia, it has become possible to focus on other aspects, including other forms of haemophilia, symptomatic carriers, rare bleeding disorders, people living with inhibitors, and people with von Willebrand's, all as they relate to both men and women.

75%: The estimated % of people globally who do not get adequate care for haemophilia and other inherited bleeding disorders.

In 2019 (slightly outside of 60 years) most members have access to longer-acting factor 8 and 9, a substantive change in therapy for the first time in 20 years. A couple of members have access to novel therapies. Why is this? Yes, we have a public health system, yes, we have clinicians who advocate for us – but that's not the entire picture. Access is not a given without what HFNZ brings to the table.

It is HFNZ's strong partnership with clinicians, our long-term knowledge of the health system, long-standing relationships with relevant government departments, long-term view of the system and experience of living with a bleeding disorder that all make it possible for us to have a high standard of treatment. There's always room for improvement, but thinking outside this room, we are among the lucky few.

Thank you for all being here this evening, the community living with and treating haemophilia and related bleeding disorders, to share your stories and share your memories, to reconnect with the past, and look forward to the future. Remember that alone we can survive, but together we will thrive.

HFNZ president Deon York

Dr Elizabeth Berry, HFNZ Patron

Friday 8 November, 2019. Grand Hall, Parliament.

Tena-koutou, tena-koutou tena-koutou katoa

I am delighted to see so many long-time friends and new faces at this 60th celebration of HFNZ. As your Patron, I wish to give you all a warm welcome- especially to Minister Hipkins, and WFH president Alain Weill.

My own association with the haemophilia community goes back just on 50 years, and I am proud of the way this organisation has grown into such a professional group serving the needs of not only the population with haemophilia but other bleeding disorders as well.

It was a roller coaster ride in many ways over the 30 years I was a treating physician, – often challenging and exciting, at times intense, demanding, and sometimes very sad. I'd like to share thoughts about the events which I think have had most influence on haemophilia care over the years.

The first was the establishment of the **Haemophilia Centre** at Auckland Hospital in 1974.

When I arrived in 1970, patients were scattered throughout general medical wards and mainly looked after by the Blood Transfusion Service who provided the only treatment available – plasma and cryoprecipitate. Believe it or not, donors were still being encouraged to run around the Domain, up and down stairs and even given adult magazines as exercise was known to increase factor 8 yields! We've certainly come a long way since then!

Formation of the Centre was a collaborative effort between the NZ Haemophilia society and treaters. With a fulltime nurse, haematologist, and other interested specialist staff we were able to offer comprehensive care. Then, as small volume concentrates became available, self-therapy at home became practical. Freedom at last! The emphasis had shifted to quality of life!

Within a short time our nurse was also fielding phone calls from patients and isolated doctors from all over NZ and even abroad. The days of long and painful waits in A and E were over. The Centre serves a much wider function though – it is a hub, a place to meet, a place of belonging

The first **haemophilia family camp** held in 1976 in Auckland was the second milestone. It was an initiative of the Goodwin family in answer to the many requests for information and support, especially from geographically isolated families – 40 people attended. We self-catered, parents relaxed and found friends, Boys met others like them and sisters learned that others had annoying and time-consuming brothers too! There was a mix of education, fun, learning to self-inject confidence building and networking.

It was also a great opportunity for staff to see families out of the hospital. My own children loved being there too, and still ask about their mates.

This format has continued to work well ever since and as you know there are now many focused and smaller workshops and weekends for different groups. It is heartening to see that there are enough older men still alive, to warrant their own group! I still think this camp model is the best way to educate and support families and to get the most value from visiting experts. I certainly feel that it is one of the most important things I have been part of. But most of you know all this already and share my enthusiasm judging by the many happy photos in Bloodline! Incidentally, NZ pioneered the family camp concept.

The next important subject is the **World Federation of Hemophila**. Feeling isolated and having a growing pile of questions, I took myself off to the WFH Congress in Kyoto in 1976 to try and find some answers and make contacts for the future.

It was a great joy and relief to be able to meet experts in all aspects of haemophilia care including product suppliers as well as people with haemophilia, and all together in one place. Colleagues from abroad were particularly helpful always posting a prompt reply, very important in the days without email.

The WFH became even more important to us during the Aids crisis providing up to date information and support and later, help with political lobby. It is a unique and important organisation serving the whole world and I am proud of NZ's contribution in recent years, especially from Deon.

Then comes topic 4, the disasters of **viral infection** in the blood supply. HIV in 1983 and Hepatitis C finally identified in 1992. Deon has commented on these, so I won't dwell in detail except to say that it was devastating for families and enormously stressful, worrying and a busy time for everyone including staff. Memories of the many studies done, trips to Wellington, reports and letters written have faded but those of the people have not.

They were courageous, resilient and often had wry humour. Some took the opportunity to follow their dreams and I particularly remember Tony who left law school to run a very successful comic shop. I suspect he may have also maxed out his student loan! Tim off to ride elephants in Thailand, Kevin upgrading his beloved Holden and exiting to the tune of Look on the Brightside of Life. They were great guys. I'd looked after them all for many years. To me, they were like extended family – It was hard.

My last milestone is **Recombinant Technology** and I am happy to hear that almost everyone is now on these products and currently being transferred onto longer acting ones, which will mean fewer injections.

With these excellent products and coordination of care through the National Haemophilia Management Group, I believe Haemophilia treatment has advanced substantially. The collaborative involvement of key players – patients, government officials and medical staff has proven highly effective and is surely the way forward.

Is there any need for the Foundation to continue? Definitely yes. Education will be even more important as people have less contact with the Centre and less ability to recognise a bleed. New babies will be born and there are continuing issues yet to resolve. There will always be a need for a political presence too.

These advances would not have taken place without the input of HFNZ and the international backing of WFH creating a positive and powerful factor in overall haemophilia management.

As I look back, my memories are increasingly about people. The many contributions you have made and the amazing courage, resourcefulness and your determination to live life to the fullest – Tim who was limited by inhibitors became a champion war gamer, Jack the intrepid sea kayaker, Mike who walked after months in bed all because he did his exercises every day. And there are many more

Care for people with bleeding disorders has never been better. I have greatly enjoyed my association with the haemophilia community and look forward to further progress in the future such as gene therapy

Affected people can expect to live a full and happy life and the future looks good. Respect, cherish and enjoy your good fortune.

HFNZ Patron Dr Elizabeth Berry

Mike Carnahan: Former President, HFNZ

Friday 8 November, 2019. Grand Hall, Parliament.

Rāhiritanga (greetings)

I want to acknowledge and pay tribute to the progress made to haemophilia care and treatment, in one lifetime – over my 73 years.

I was born in 1946 - April 1947 was my first bleeding episode, with the classical prolonged bleeding from the mouth after a fall. It was four years before my clinical notes referred to the principle disease as haemophilia – although one reference was – "suffers from intermittent haemophilia". A referral in 1950 loudly described the desperation of treaters "any effective treatment is unlikely, but on the other hand I am desperate with a young child who will likely die from bleeding. I feel it unlikely that any effective treatment is possible but, in view of his age, one feels that all possible should be done." I was duly sent off to Wellington Hospital.

In this era of 70 years ago – all the clinician had in their diagnostic kit was observation and family history. There were no useful diagnostic tests available. My clinical notes only record bleeding time and clotting time. Similarly, there were no treatments available, other than whole blood. I was aged 13 before a diagnosis was made.

But by the 1960s I was benefitting from availability of blood plasma which did improve the treatment for both factor VIII and IX. During this era the first concentrates of the factor proteins we were all short off, began to appear. That was cryoprecipitate.

The big stride forward came in the 70s with the availability of plasma sourced, commercially manufactured, medium purity, concentrates. Then along with developing degrees of prophylaxis - days off school, days of work, days in bed and on the couch became significantly reduced - even some minor surgeries were undertaken.

These trends were developed in the early 80s, although increased physiotherapy was added to try to develop muscle to protect significantly damaged joints. But this golden age of the early 80's quickly disappeared in 1983 with the emergence of Human Immunodeficiency Virus (HIV). Overnight the treatment of bleeds by plasma based medium purity commercial products disappeared as HIV developed and resulted in 29 NZ people with haemophilia becoming infected. Florence Nightingale's principles of nursing that individual treatments must not further harm the patient were dashed.

Once a diagnostic test for the presence of HIV was developed, along with a more rigorous scrutiny of blood donors and the testing of every donation prior to manufacture, we began to use our commercial products again. It was only after this event that we learned how sparing the checks and balances had been and how little the Ministry of Health knew about blood and its use.

But there was a second virus lurking in every country's blood supply – hepatitis C virus (HCV). This disease was known even during the era of WWII. Probably more than 90% of users of what was by then termed medium purity concentrates, became infected with HCV. The result was the infection was passed on to partners and more than 40 people with haemophilia died where this virus was a direct or secondary cause of death. The rest of us underwent multiple attempts to clear the virus at an enormous but unquantified cost for the health system and the individual. The damage was made worst by inept political decisions and again sloppy ministerial oversight.

In 1998, New Zealand introduced prophylaxis for all newborns with haemophilia, which required a significant increase in the uptake of recombinant product. I became one of the last people with haemophilia to use plasma-based prophylaxis therapy (albeit significantly purified by 2019 to that used in 1975), I switched to recombinant therapy in 2019, requiring an IV infusion but once per week.

So, when you consider the progress in diagnosis and treatment over one lifetime, I consider the progress has been both costly and remarkable. Haemophilia was termed the world's most expensive disease to treat. But it has also been a very expensive disease for every person so afflicted, and some more than others. We are now at the point where probably 95% of haemophilia care is carried out in the home with individuals taking responsibility for use of an expensive medicine.

I believe the primary reasons for progress in NZ over the past 60 years has been the unique model of participation and decision making crafted into Haemophilia structure from its beginning, and now used across the Haemophilia world. This cooperative and consultant format, which directly involves patients, clinicians, funders, and manufacturers when making decisions on practice and supply, must never be lost. It is also important the Foundation continue its role of educating families who have the haemophilia gene. This education is even more important now that such a high proportion of care is carried out in the home without direct clinical oversight.

I now look forward to the next era of improvement, which appears to be gene therapy.

Noho ora mai (stay well, goodbye)

Tēnā koutou (thank you)

Former HFNZ president Mike Carnahan

19th Australian Conference on Haemophilia, vWD, and Rare Bleeding Disorders

Sue Ellis: Along with the four Outreach Workers, I recently attended the HFA conference held in Manly, Sydney. This was very well attended by people including patients and their families, clinicians, pharmaceutical representatives, and other professionals working in the bleeding disorder community. The conference included a dinner where I was fortunate to meet and sit with the President of the HFA, and everyone seemed to enjoy the dancing afterwards.
There were a number of excellent presentations over the two days.
This report covers the sessions the team and I attended.

Improving Outcomes: What Has Been Achieved in the Treatment of Bleeding Disorders?

INTRODUCTION AND CURRENT TREATMENTS: DR LIANE KHOO

BY SUE ELLIS

Dr Khoo, as Chair, gave a background to the history of BD treatments from 1840 (with George Firmen who explored genealogy) to 1965 with FVIII, and then the ongoing evolution of products to where we are today looking at the realities of gene therapy. In Australia, there are 6000 people on 10 clinical trials.

PERSONAL EXPERIENCE: DR SIMON MCRAE AND JOHN

BY ROSS PATERSON

Simon has a background working in Haematology for the past 15 years and is currently Director of Haemophilia Treatment at the Royal Adelaide Hospital. Dr McRae briefly outlined the increasing evidence that prophylactic treatment has resulted in much improved outcomes for patients. In addition, in Australia, patients involved in clinical trials are helping to change the targets for improved outcomes and optimising outcomes for all patients.

John, a 53 year old patient, whose current haematologist is Dr McRae, was able to provide snapshots of the impact that his bleeding disorder has on him, and the evolution of the treatment in his lifetime. John was reliably informed that his first bleed occurred when he was ten months old. Subsequently, he was diagnosed with severe haemophilia A, and treated with cryoprecipitate. In those earlier days, John described a typical treatment from the time of leaving for hospital being a four hour round trip, which typically meant more damage done to his joints due to the time it took for treatment to commence. John described experiencing excruciating pain during his treatments of the 1960s and 70s, and he did not want to go the hospital. He described feeling like a pincushion at the various failed attempts to find a vein for his treatment. One impact of the stress he experienced was hives all over his body, and he was prescribed antihistamines to alleviate the condition.

John described frequent bleeds to his right knee and left elbow during this period. He spent many months in hospital and at times had leg in plaster, in traction, and he was also required to wear callipers on his release from hospital. He was not allowed to participate in sporting activities and his education was severely interrupted by his stays in hospital. In the 1970s he moved to using freeze dry cryoprecipitate. Then, in the 1980s, a huge break through occurred when he was able to infuse himself at home, resulting in much quicker treatment and no further hospital admissions. It also meant he was able to enjoy camping and fishing excursions for the first time, activities that he enjoyed immensely.

In the early 2000s John contracted HEP C through bad blood. He was unsuccessfully treated with interferon. He described this as a very dark period, affecting both his working and social life. Later treatment included ribavirin and interferon and these were successful in clearing the hepatitis. Subsequently John started going to the gym. In 2011 he commenced taking adnate prophylactically, initially on a trial, and described this as a life changer. This ultimately switched to an extended half-life product. Gym activities now include pump, circuit training, and boxing. Last Thursday John commenced a gene therapy trial and he has an optimistic view of the impact of this treatment for himself.

OPTIMISING PATIENT CARE THROUGH PK ANALYSIS (WAPPS): PROF ALFONSO IORIO OF MCMASTER UNIVERSITY IN CANADA

BY LYNNE CAMPBELL

Prof Alfonso Ioro's presentation focussed on optimising individualized treatment in order to manage bleeding in Haemophilia through PK-based monitoring, rather than addressing bleeding based on phenotype.

In order to calculate the curve to show how effective treatment is in a patient, and how long protection lasts, optimal dosage can be worked out by having any two out of the three measurements dose/interval/level achieved. Two of the three are crucial in order to calculate the trough, or the dose. The resulting curve is useful in identifying the level of protection provided against bleeding for the patient.

Treatments with "enhanced" PK benefit from profiling just as much as shorter half-life (SHL) treatments.

With EHL (extended half-life) treatment, the dose rate can be adjusted to maintain the trough at a level that protects against bleeding. As Takeda's PROPEL study with Adynovate found, it is possible to maintain a clotting rate as high as 10%. However, clinically, to be treating every other day would defeat the purpose of being on an EHL regime when the trough can be managed differently.

The app My WAPPS was strongly promoted in this presentation as a way to monitor factor levels in real time, including estimating future levels, receiving reminders when it is time for an infusion, and receiving notifications when factor levels drop to the low zone. This is the app used in Canada and is similar to Takeda's app My PK Fit, which links by email to your HTC.

Essentially, better bleed control results when your individual pharmacokinetic (PK) study is monitored. Your personalised PK records then calculate your treatment regime, informs decision making about treatment options such as EHL products, and optimises decision-making.

Variability: To be able to maintain an individual's factor level over time is key, however, variability cannot always be explained.

Between 0-2 years of age the PK changes, thereafter PK becomes linear and linked to weight and age. The WAPPS app takes into account weight and age so therefore helps in tracking treatment requirements.

Tolerisation: During tolerisation PK is repeatedly measured.

Bleeding control is also influenced by the other clotting factors in the blood so factor level correlation can also affect over-all bleed control.

Addressing the Challenges of Inhibitors

A CLINICAL OVERVIEW – DR HUYEN TRAN

BY LYNNE CAMPBELL

Three factors influence the formation and treatment of inhibitors

1. Patient Genetics – which includes the type of mutation, the genotype, race and family history
2. Patient Environment – which includes the age of first infusion and immune system challenges
3. Treatment – which involves the type of factor and quantity.

It is important to note that non-severe patients can also develop inhibitors.

High titre inhibitors are much more difficult to tolerise. For the patient it is a lifetime of persistence and endurance.

Emicizumab is an antibody technology administered subcutaneously, and has become the preferred treatment for Factor VIII inhibitor patients. Other clinical trials have investigated Fitusiran and tissue factor pathway inhibitor as treatments to address bleeding in Factor IX patients with inhibitors.

An example was given of a young Factor VIII patient where tolerisation hadn't worked, but with the introduction of Emicizumab there was a dramatic improvement. The same was found to be true in adults. Emicizumab has also been shown to work to reduce bleeding with or without an inhibitor in Factor VIII patients. Over time, the proportion of inhibitor patients with zero bleeds has increased.

Careful planning for surgery is required for patients with chronic inhibitors receiving Emicizumab.

Inhibitors remain an important morbidity among people with Haemophilia.

DISCUSSION AND DEBATE ON IMMUNE TOLERANCE INDUCTION THERAPY – DR CHRIS BARNES AND DR JULIE CURTIN

In this section of the presentation two medical colleagues debated whether Emicizumab should be a front line treatment for (all) patients with Haemophilia and Inhibitors. They were encouraged to argue the opposite to what they believed where possible in this debate.

Consideration was given to the fact that not all inhibitors, or patients, are the same and other new treatments are in production to address peaks and troughs in the management of bleeding. The reasonably good efficacy of bypassing agent therapies was addressed, along with the justification for immune tolerance therapy. Given the relatively common development of inhibitors in Haemophilia A (33% in severe Haemophilia and 13% in those with non-severe A) coupled with the reduced quality of life for those who develop problematic Inhibitors, it became increasingly difficult to defend not administering Emicizumab.

PERSONAL JOURNEY – ANDREW

In the third section of this presentation, 25 year old Andrew Selveggio took the audience on a journey through his personal medical history from child to adulthood as a patient with severe haemophilia A with Inhibitors.

He grew up in an era where improved treatments were always on the horizon or just around the corner, but in reality out of reach. His parents were powerful advocates for him and taught him how to advocate for himself.

His medical stats were alarming – all bleeds and their nature were itemised in sequence as he progressed from diagnosis with severe haemophilia A as a 2 year old, then to a 4 year old with inhibitors, through to adulthood. He had no inhibitor treatment until receiving a bypassing agent in 1997, when he was 11. He was overweight and wheelchair bound from age 5-18, had numerous major surgeries and infections, and a total of 8 years as an inpatient.

He is based in Melbourne and in the care of Alfred Hospital. For 3 ½ years Andrew has participated in the Hemlibra Haven 1 trial which started in 2016. His father was so happy with this subcutaneous treatment – no more venous access! The haemophilia care that has long been promised is now here. (i.e. zero bleeds).

Andrew learned, at age 20, the importance of keeping moving, of not being complacent, and the need to be self-managing. He lost 30kgs/66lb. Although Andrew has had two knee replacements, bilateral ankle fusion, and physiological issues relating to chronic arthritis in old target joints, his life is now completely different.

Hemlibra has been life changing for him and his capabilities. He regularly attends the gym where he is a personal trainer.

Andrew has undergone a big adjustment in terms of his mental health (the patient's experience). He still has an inhibitor.

He can now plan for his future. He is fit, slim, married, has long-term career goals, plans to travel the world, has bought some land, and ultimately wants to build his first home.

Getting Older

PREETHA JAYARAM: PATIENT VOICES

BY SUE ELLIS

"Community Voices" is a getting older project with the aim to better understand and respond to larger numbers of older patients with bleeding disorders. The needs assessments include interviews with counsellors and community members to understand their needs. Comments reflected that of the general population: financial and social security, family, retire and scale down work. A survey and focus groups were undertaken, collecting age-related data.

Concerns: joint stiffness and pain, fatigue, depression, ending up in an aged care facility, have no direction; partner takes the burden of care, lack of dexterity, for example doing my buttons up.

PENNY MCCARTHY: TREATMENT AND CARE AT HAEMOPHILIA TREATMENT CENTRES

BY SUE ELLIS

People with bleeding disorders are now at risk of general age-related disorders unrelated to haemophilia, that is, same problems as general population. The 2010 European Haemophilia Consortium called for better social support for aging patients – e.g. an enhanced multidisciplinary team approach. Seeing different presentations e.g. joint issues from gardening or lifting grandchild. We are a product of our own success with increasing wellness, older patient numbers increasing, but staffing numbers static - work efficiencies made through technology. Greater numbers of people with cardiovascular disease, blood pressure monitoring. People with bleeding disorders have higher prevalence of increased blood pressure and obesity, along with an increased risk of developing heart disease, type 2 diabetes, and certain cancers. They often have difficulty finding veins, risk mild to moderate inhibitors with increased exposure to factor (e.g. at age 5, 15, 50 and 75 risks are 5%, 6%, 10% and 12% respectively), require injections for joint pain, and experience decreased eyesight, and reduced mobility and dexterity.

GREG BLAMEY: MANAGING INDEPENDENCE AND KEEPING UP WITH EXERCISE

BY ROSS PATERSON

Medical management of haemophilia has seen rapid advances in recent years with the advent of extended half-life products and novel therapies. However, the impact of these therapies is less likely to be realised by older adults; and the ability to provide greater strength, stamina, fitness and therefore the maximised quality of life remains the domain of exercise and physical activity. This presentation focused on how the incorporation of exercise into a lifelong routine promotes independence with reference to issues faced by people with haemophilia as they age. The concepts of ageing and enhanced musculoskeletal fitness are both undergoing revolutionary change now, with respect to historical perceptions of what can be achieved by people with a bleeding disorder; a paradigm shift that stands to benefit all people with haemophilia across their lifespan.

Greg provided a number of examples of the impacts of excess weight on our joints, in the context of ageing. These include:

- For every increase in body weight by 1kg 9–13 % risk of joint damage
- For every increase in body weight by 5kg, 25% risk of joint damage.

In addition, for people with mild/moderate haemophilia the risk of inhibitors grows with an increase in body mass, and raises the question of how long these people can continue with prophylaxis.

Maintaining exercise as we get older can make a massive difference. Ageing is contextual – i.e. a combination of chronology and lived experience. In the context of realistic goals, we are never too young to start exercise.

Recent statistics illustrate that:

- 74% of children aged 5–12 years have insufficient exercise
- 92% of 13–17 year olds undertake less than the recommended amount of exercise
- 52 % of 18 – 64 year olds have less exercise than that recommended with 70% having less than an ideal level of strength
- 75% of the over 65s fall short of the recommended amount of exercise.

It is obviously extremely unhelpful for people with impaired mobility to hear that "he is 20 with the joints of a 60 year old" or "he is old before his time". A rationale for taking up exercise is to maintain independence. If we have arrived at a state of dependence on others for daily living, this would appear to be irreversible. However, if we have moved to a pre-dependent state, we can regain independence or continue to maintain independence.

Fear of what can happen if we take up regular exercise can be unlocked if we talk to our physio. The evidence tells us that we need at least 100 hours of exercise per annum. In addition, the exercise components need to include a number of elements to improve the following: flexibility, endurance, strength, and balance. Not just strength. An individual's programme needs to be regular, weekly, and ongoing.

Getting Older (continued)

MARCIA FERN: INNOVATIVE PROJECTS IN AGEING

BY ROSS PATERSON

Marcia Fern from the National Ageing Research Institute presented information that was not specific to the bleeding disorder community.

Due to improvements in medical care, the life expectancy of people with haemophilia and other bleeding disorders is increasing. Getting older can affect a person's health and wellbeing, and older people are more likely than younger people to have multiple long-term health conditions.

This increased life expectancy may bring with it many other issues or concerns that the person with bleeding disorder may not have experienced in the past. They now not only have to consider their bleeding disorder, but also other medical comorbidities that can come with ageing.

Marcia examined some of the challenges of getting older. These included:

- Loss of positive reinforcement
- No longer able to do things that have been important to us
- Loss of independence
- Being a carer
- Loss of spouse
- Feelings of being left behind

So what is the key to ageing gracefully? What is important as we age?

Being meaningful and productive are important.

The latest evidence re. dementia risk indicates that in middle age hearing loss, hypertension, obesity, smoking, social isolation, and diabetes are all high risk factors leading to dementia. If we treat these issues then the risk diminishes.

What can we do?

Strategies to assist in successful ageing

Treat them to reduce risk:

- Depression is the most common mental health issue in the elderly. Befriending the elderly (befriending programmes for peer support), has been identified as a major means of alleviating this depression.
- Falls are the single greatest reason for hospital admissions in the over-60 age group. Effective falls prevention programmes, exercise to improve muscle strength, balance, and cardio vascular capacity are keys to assisting here. Ensuring that eyesight is maintained as well as possible, i.e. wearing prescription glasses and having treatment for cataracts.
- Volunteering/social programmes such as Men's Sheds, and some pain management strategies.

Looking After Children Who Have a Bleeding Disorder

BY AMBER MAIHI

Chair: Robyn Shoemark

Speakers: Robyn Shoemark, Dr Emma Prowse, Anne Jackson, Alison Morris, Johanna Newsom

The session opened with Robyn Shoemark from The Children's Hospital at Westmead. She spoke about the importance of encouraging independence and overcoming barriers to self-infusion. A big part of this is normalising bleeding disorders from a young age, as young as two years old. This can include talking them through the treatment process, detailing each step so they become more familiar. Allowing them to interact with their treatment, for example putting on the tourniquet or asking them what comes next. The goal being that the child is confident to self-infuse by about year 5 in school, as this is around the time that they are gearing up to head off to their first school camp.

A section of Robyn's presentation was dedicated to the difficulties of transition to self-infusion, including needle phobia, separation anxiety, and PTSD (post-traumatic stress disorder). These are very common and are not removed easily. Not one size fits all for families with bleeding disorders and the most important thing to remember is patience. Taking time to understand your child's needs will make all the difference.

Dr Emma Prowse spoke about the impact of chronic illness on families focussing on Haemophilia and other bleeding disorders. She highlighted the connection between bleeding disorders and self-identity in children noting that a majority of children between the ages of 4 to 10 find it difficult to separate their conditions from their own self-identity.

Dr Prowse emphasised the point that long-term chronic illness can lead to increased stress in patients and their family members. This can also have a significant impact on their social and emotional development and young children and adolescents. During this time they are beginning to develop empathy and concern for others as emotional understanding improves and can start to feel a sense of unfairness or injustice if treated differently from peers or siblings. These are some important signs or behaviours for parents to look out for and when psychosocial intervention may be needed. These are:

- Changes in sleep and appetite
- Withdrawal from friends and previous enjoyed activities
- Headaches and stomach aches
- Getting in trouble at school
- Increase in rule challenging behaviours
- Outbursts of anger

Some key points when supporting a child to recognise their own self-identity, that they are not just their illness, are:

- Increasing their understanding about the illness
- Communicating health related worries
- Allowing them to interact with treatments to normalise this process
- Finding enjoyed activities that boost peer relationships
- Advocating for your child. You are the expert about your child.

School attendance is also paramount during this time, as peer and group interactions help to shape a child and their outlook on life. If they are present, they are less likely to feel excluded and will have more confidence. Helpful tips for overcoming these obstacles are:

- Finding balance between autonomy and independence with ongoing monitoring and support
- Developing self-efficacy and assistance to maintain adherence to treatment
- Understanding the importance of trust.

360 Degree on Clinical Trials

ALICIA: A FAMILY'S EXPERIENCE OF A CLINICAL TRIAL FOR CHILDREN

BY NICKY HOLLINGS

Alicia's six-day-old baby had jaundice and was having regular heel prick tests from which the clinicians determined that he had severe haemophilia A.

He is now part of an extended half-life trial. From the beginning of the trial they had a team that they trusted and knew. However, part way through the trial they had to change to a new team. Though ultimately beneficial, the change was very stressful. They had fears at time about whether their child was being used as a guinea pig.

They were able to come to terms with the trial and the treatment due to the evidence of the effectiveness of the treatment when used on adults, and because they had the support of the treatment team.

There were several benefits from the new treatment. One less needle a week means at least 52 fewer treatments per year. In addition, Alicia's husband learnt early how to administer the treatment, so he was able to share the regular treatment, as well as when an injury took place. However, they weren't prepared for how difficult the hospital visits were.

When discussing clinical trials at the end of the section, people in the audience expressed concern that when they had been on a trial, they weren't able to continue using the treatment at the end of the trial. In addition, if the patient lives 3 or 4 hours away from the hospital, it becomes difficult to adhere to the rules and guidelines of the trial.

HAMISH: AN INDIVIDUAL'S EXPERIENCE OF A CLINICAL TRIAL

BY NICKY HOLLINGS

Hamish discussed how he was a non-compliant patient, how he struggled to remember to do treatment. When asked whether he would like to go onto a trial, he took the opportunity.

Since going on the trial he hasn't had any breakthrough bleeds. That has meant that he has been able to live his life without worrying, which has created independence and hope.

DR SIMON BROWN: THE ROLE OF YOUR DOCTOR IN A CLINICAL TRIAL

BY SUE ELLIS

When it comes to clinical trials, doctors always have to ask themselves; do I have the time and resources, and what will be the impact on other services. There are always systemic and organisational barriers; for example time and resources, space, storage, staff, ethics and consent processes, documentation, trial design, impact on clinical practice, and training and recruitment.

Good clinical practice when conducting trials is based on the Helsinki Declaration of 1964 on human experimentation for the medical community. Clinicians need to assess the benefits, justify the risks, ensure the rights and safety of subjects are paramount, and follow the protocol for approval of an independent ethics committee. They also need to plan for hurdles such as adverse event and protocol deviations, such as missing a visit because the subject is traveling.

The benefits of clinical trials include the development of new therapies, learning opportunities, societal benefits, and adding to the clinicians' training and experience.

DR ALFONSO IORIO: PATIENT REPORTED OUTCOMES TO IMPROVE THE VALUE OF CLINICAL TRIALS

BY SUE ELLIS

What outcomes information should be considered in clinical trials? With new therapies, should we be looking at new goals? Measuring the overall benefit over the whole lifespan is directly related to consistent collection and reporting of relevant and well-specified data. It is important to understand what matters to patients, and to measure outcomes that encompass the whole cycle of care. Value is a matter of perspective. As Einstein said: "Not everything that can be counted, counts. Not everything that counts can be counted".

There are some limitations to clinical trials of bleeding disorder treatments. Blind trials are rarely done for haemophilia medication, because all participants still need treatment, and need to provide anecdotal data. In addition, access to accurate quality of life data over the period of the trial can be confounded by the disability paradigm. The development of long-term joint damage requires long-term observations. Some of this data can be accessed from the PROBE (Patient Reported Outcomes Burdens and Experiences) study, a long-term patient developed and designed questionnaire project. The PROBE data can be compared with non-haemophilia benchmarks across countries, measuring change between trial participants and the PROBE cohort.

Reproduction and Family Planning

GENETICS, REPRODUCTION AND FAMILY PLANNING: A/PROF KRISTI JONES – GENETICIST, AND LUCY KEVIN – GENETICS COUNSELLOR

BY LYNNE CAMPBELL

The presentation that followed the personal journey stories covered the following aspects of inheritance:

- How did this bleeding disorder happen?
- Could other family members/future children have a similar bleeding disorder?
- Gene testing other family members?
- Can we test for the bleeding disorder during pregnancy?
- Is there a way to avoid having a pregnancy with the bleeding disorder?

Genetic counselling is a communication process, which aims to help individuals, couples, and families understand and adapt to the medical, psychological, familial, and reproductive implications of the genetic contribution to specific health conditions.

Through the genetic counselling conversation, clients learn about what to expect given their new circumstances. Individual experiences differ and no judgement is assigned to the clients as they work through this process and come to make and understand any future reproductive decisions.

The client has an opportunity to learn in detail how the clotting cascade operates, how the clotting factors form, and of the protein mutations from which Haemophilia A, Haemophilia B, and Von Willebrand Disease are derived. This provides an insight into how their particular mutation came about.

Family history and inheritance patterns are investigated to determine whether this is a new genetic change, or has been inherited from one or both parents. Is it a sex linked inheritance pattern, such as in Haemophilia A and B, or via somatic inheritance, as in Von Willebrands Disease? Alternatively, it may be the result of skewed X chromosome inactivation, resulting in a female, genetically and clinically, showing moderate to severe haemophilia and bleeding. This discussion inevitably leads to considering the implications for other family members.

In order to detect the gene mutation accurately, gene testing should only be done through your HTC. Caution was recommended regarding lower level reproductive screening agencies. The timing of carrier testing was also touched on. Is it better for a young girl to grow up knowing, or to allow a young adult to make that independent adult decision given the risk of altered self-image. Essentially the message was not to test growing children who had no symptoms. However, it is important to note that factor levels alone do not determine whether a person has inherited the haemophilia gene. Open communication and understanding throughout life stages inform family members and prepare individuals in advance for the results of testing when the time is right.

It takes time for the results of genetic testing to come back, so requires additional planning either before or as part of family planning.

Carrier Testing in Healthy Individuals:

- Can't rely on factor levels alone
- Genetic carrier testing can clarify chance of having an affected child and may inform pregnancy management
- Informs whether there is a need for genetic testing in other family members
- Genetic carrier testing for obligate carriers.

Genetic testing can also be carried out during pregnancy. The decision to test or not is personal.

- The NIPT test (Non Invasive Prenatal Testing) is a simple blood test that determines the sex of the baby from analysis of free floating foetal DNA in the mother's blood, but not the presence of an altered gene. It is performed from 10 weeks. Results come through quickly.
- Chorionic villus sampling (CVS) is more invasive so carries a small risk of miscarriage. It is performed later, at 11-12 weeks of pregnancy, but genetic testing for familial mutation can be performed on sampled tissue.
- Amniocentesis test is done slightly later, at 15 weeks.

The timing and reason for testing determines which test is most suitable for the individual and undertaken.

To avoid having a pregnancy affected by a bleeding disorder some couples elect Preimplantation Genetic Diagnosis (PGD). Success depends on maternal age, co-existing fertility problems, and the genetics of the condition tested for.

There is no right or wrong answer to reproductive decision-making and the pros and cons need to be weighed up by the individuals concerned.

The Psychosocial aspects of decision-making may be influenced by:

- Experience and perception of bleeding disorder
- Religious/moral beliefs
- Feelings of guilt/responsibility
- Consideration of impact/views of partner/other family members
- Reproductive history
- Economic/practical considerations

A Healthy Life for All Ages

BY ROSS PATERSON

JULES AITKEN: MANAGING A HEALTHY DIET AND WEIGHT

Jules provided an overview of how we rate in the health stats. She reminded us that being overweight or obese can cause a significant disease burden on joints already vulnerable to inflammation and haemophilic arthropathy.

- 35.5 % of Australians are considered overweight and 28% of New Zealanders. (Recent stats provide for a higher tolerance of weight in the large Polynesian population because of their greater bone density).
- The numbers of overweight and obese people is on the rise in both Australia and New Zealand
- Our obesity levels in the two countries are second only behind the United States.

What are some of the consequences of these rising obesity levels?

- A sharp rise in cardio vascular issues for these people
- Larger numbers of people presenting with diabetes
- An increase in people having musculoskeletal problems
- Decreased range of motion of joints, accelerated loss of joint mobility
- Increased chronic pain
- A noticeable rise in some forms of cancer
- Childhood obesity leading to higher risk of obesity throughout life
- Increase risk of osteoarthritis
- For men, reduction in testosterone levels and poor self-image jointly causing loss of libido
- Obesity rates in haemophilia community are comparable to the general community
- A condition known as non-alcoholic fatty liver.

Even 1kg of weight loss can amount to 2kg less pressure on our bodies.

The differences between weight and health:

- Simply put, it is the consumption of more calories than we expend
- Obesity is about energy balance
- Men are less likely to perceive themselves as obese – just overweight
- Post-menopausal women who experience weight gain and find it more difficult to lose weight
- Taking anti-depressants can increase weight gain, and this in turn adds psychological stress and a lack of self esteem
- The use of steroids can impact on increased likelihood of diabetes and thyroid problems
- Many overweight or obese people do exercise regularly
- Many overweight or obese people experience sleep apnoea
- When we provide comfort to children – we can slip into the habit of providing food – as this is a natural form of comfort. We need to be careful what we give and the amount.

Maintaining a healthy weight reduces the burden on joints and long-term health conditions. Obesity is preventable, but losing weight and maintaining a healthy weight for many is not as simple as 'exercise more and eat less'. Physiological, genetic, environment, socioeconomic status, and psychology all play a part in energy balance. Hormones affect hunger, satiety, and fat storage. Information about how to make smarter food choices and weight loss treatments are available to assist with weight loss and maintenance.

Start the conversation.

Why do I want to lose weight? Is it painful joints, current health status, genetic risk factors, or something else? We now understand that losing five to 10% of weight is clinically meaningful. The dilemma about losing weight is doing it so that it is sustainable. There is more involved than eating less and exercising more. Weight loss causes the body to alter protein levels. We need to monitor the types of food that we eat. 45- 65 % should come from carbohydrates. We need to be aware of the strong influence of sugar in processed foods. For example, muesli. The amount and type of fat we eat is another factor to be considered. Eat the best and leave the rest. Remember not all calories and food are equal. The calorie intake for an adult male 66 years plus should equal 6.3 x their body weight in pounds. In kilojoules, that's 58 times their body weight in kilograms. A useful app is My Fitness Pal.

GREIG BLAMEY: EXERCISING SAFELY AT ALL AGES

In Greig's practice, at the Department of Physiotherapy in Winnipeg, he advocates that people with bleeding disorders exercise safely at all ages. He and his colleagues recommend physical activity for all people with bleeding disorders. They take exercise to the level of prescription – i.e. not just recommending it but rather prescribing exercise as part of treatment.

The Factor Revolution.

Factor treatment has made a huge difference to the affected population. However, treatment must include more than factor. Why not include a physical prophylaxis. It is important to focus on personalising care, because one size doesn't fit all. Bleeding disorders are a psychosocial issue too, and there is an argument that we should create funding to facilitate more physical activity, rather than relying solely on pharmaceuticals.

Greig advocates using a personal trainer, although he acknowledges that this is not cheap. However, he cites the potential savings in factor usage, increased productivity, and overall health benefits. For example, one patient now uses 3500 IU/week as opposed to 6000 IU/ week.

There is the potential to be a lot more creative in devising individualised training programmes and carefully targeted physical programmes. The clinic where Greig works utilises the expertise of a physiotherapist alongside that of a certified personal trainer and cooperation and operational funding from the Canadian Haemophilia Society.

TIM DESCRIBED A PERSONAL EXPERIENCE OF MANAGING HAEMOPHILIA CHALLENGES AND KEEPING FIT AND WELL

Tim described being impacted by severe haemophilia A, and that he uses prophylactic treatment four days per week. He outlined the desire to look after himself as well as possible and to push himself via mental challenges and a healthy lifestyle. In his working life, he is a paramedic, a role that has a high injury rate, so looking after himself is vital.

For ten years Tim played basketball for fitness and fun, but eventually decided that he was getting too many ankle injuries and he needed to find an alternative physical activity for maintaining physical wellbeing. For the past five years Tim has undertaken weight and resistance training which has resulted in a large increase in his muscle mass, huge benefits to his wellbeing, and reduced his factor use. He emphasises listening to your body and not your ego, and that if you do that, the risk of injury or a bleed is minimal, and, in the context of his whole lifestyle, decreases overall. The key, to his mind, is tailoring training to what works for you.

Dr Tim Sharp (Dr Happy): Challenging the Status Quo

BY LYNNE CAMPBELL

THE 10 HABITS FOR HAPPINESS

Dr Tim Sharp is Australia's very own 'Dr Happy', at the forefront of the positive psychology movement as founder of The Happiness Institute. He offered his 10 Habits for Happiness:

HABIT 1: CREATE YOUR OWN DEFINITION OF WHAT HAPPINESS REALLY MEANS FOR YOU

Happiness is individual; it changes over time and relates to meaning and purpose. It is necessary to have a plan in order to achieve it.

Even when aiming for happiness it is natural to feel frustrated, angry etc.

When plotting overall wellbeing against a positivity ratio, there is a period of flourishing which is followed by a plateau. This region is known as the flourishing range and the zone where overall wellbeing crosses over to plateau is known as the tipping point. Ideally, the positivity ratio between these two is around 3:1.

HABIT 2: SET AND WORK TOWARD MEANINGFUL GOALS

Make your goals SMART.

S.M.A.R.T goals are specific, measurable, achievable, realistic, and time-bound, so should be reviewed regularly.

HABIT 3: LAUGH, PLAY AND HAVE FUN!

When fun and play are used appropriately, they can be powerful forces for good.

Now it is recognised that positive emotions serve an important role in seeing new opportunities. Through opportunities and experiences, we become better at building on our internal and external resources.

We tend to postpone happiness. This is sometimes known as 'the tyranny of when'.

Less desirable activities can be enhanced by including or adding something that makes the activity more fun, for example exercising while listening to music.

HABIT 4: JUMP OUT OF AN AIRPLANE

To take on a scary challenge can give a person strength. If one can overcome the fear, and the fear becomes a thrill, then imagine what else the person can do.

Experiences are powerful; we can't protect ourselves, or our children, from everything.

HABIT 5: EXERCISE YOUR RIGHT TO BE HAPPY

Exercise is a mood enhancer. To move our bodies more is also a stress buster.

The benefits of exercise are further enhanced by eating real food and not processed food.

HABIT 6: SLEEP YOUR WAY TO THE TOP

Sleep is crucial to wellbeing. You should wake up feeing refreshed. Poor quality sleep negatively affects physical and mental wellbeing. It is hard to be happy when you're tired all the time.

Insufficient quality sleep is a problem for children as well as adults.

HABIT 7: ASK FOR HELP

Men are particularly poor at this. We can't be our best on our own.

The interaction between patients living with chronic pain and their spouses is very important. There are three types of responses by a spouse to the person in pain:

a. Punishing
b. Overly solicitous, i.e. doing too much for the person
c. Helpfully supportive, i.e. let me help you to help yourself.

This third type of response has been shown to contribute to longevity, happiness, and health.

The quality of our relationships is highly influential in living our best lives, "other people matter".

HABIT 8: GIVE HELP

Giving help, for example, through volunteering is very powerful for our own happiness and health. It is provides a win-win to all concerned.

HABIT 9: CHOOSE YOUR FOCUS

The focus of mainstream media tends to be on the "bad things". Good people doing good things don't get a lot of attention.

Day to day we are subject to the "cocktail party effect", i.e. given the high sensory input of all we are exposed to, we can't take in everything, so filter things out and only focus on what we think is important.

The focus of our attention needs to be helpful. We need to be aware and change our focus to the good things. To practice gratitude and appreciation works to help in rechannelling our focus and make us happier.

The VIA Classification of 24 character strengths was touched on as an aside as they are "energisers" that promote or enable wellbeing. Essentially, those who are aware of their strengths are more content and happier.

HABIT 10: THERE IS ALWAYS MORE HAPPINESS THAN WE THINK!

Everything You Wanted to Know But Were Afraid to Ask

BY ROSS PATERSON

Chair: Claude Damiani,

Panel: Greig Blamey, physiotherapist; Tim, a patient and paramedic; Zev, a patient and business owner; Dr Nalini Pati, Haematologist.

Discussion was held around the impact of vigorous sex and how this can lead to bleeds for men. Greig suggested that planning prophylactic treatment around sex was a strategy for mitigating bleeds. However another point of view proffered by another attendee at the breakfast put forward was that he would want sex to be spontaneous and not planned around prophylactic treatment.

Another issue canvassed was that of the ease to disclose a bleeding disorder to a prospective sexual partner. This was clearly a much easier discussion in the current era, whereas in the past, especially during the bad- blood era, it was a difficult conversation for men.

In the context of work or other social environments, men described often disclosing their Hep C or HIV status on a need-to-know basis, with trust being a factor in their decision-making.

Older men described the experience of poor treatment and advice in the past, but generally talked about how this has improved over time. They also acknowledged how their mothers in particular, advocated strongly on their behalf, and how in turn they needed to be able to self-advocate as they matured. For some this resulted in a variety of issues when they weren't listened to.

Musculoskeletal Challenges: Joint Care and Treatment

DR ROB RUSSO: THE UTILITY OF ULTRASOUND IN THE EVALUATION AND MANAGEMENT OF HAEMOPHILIC ARTHRITIS

BY LYNNE CAMPBELL

In this session, Dr Rob Russo reviewed imaging options in haemophilia arthropathy, discussed the role of imaging in joint care, and focused particularly on the use of ultrasound and its usefulness in deciding treatment for joint care. The session concluded with a demonstration of ultrasound of the elbow.

Imaging aims to gather information not available to the practitioner by other means. X-rays and MRI imaging certainly have their place, but ultrasound is accessible, can be done by someone who is not a radiologist, easy to perform, inexpensive, immediate, shows early signs of change, and can be done while the practitioner is actually moving the joint, so any preclinical limitations to movement are readily noticed.

The elbow, ankle, and knee are the most common sites for joint disease and functional impairment resulting from haemarthropathy. Inflammatory damage from bleeding into joints results in altered bone formation and changes to the biomechanics of joints. The purpose of imaging joints using the Haemophilia Early Arthritis Detection with Ultra Sound (HEAD-US) approach is to:

- Detect site and severity of joint damage
- Detect changes prior to irreversible damage and predict the risk of further bleeding
- Evaluate a joint and provide a tailored approach to management, then monitor the effects of treatment.

Ideally, adults with severe Haemophilia A or B should be on a 12 monthly schedule, and children seen six monthly.

In the acute setting, ultrasound is useful in the evaluation of a severely swollen joint to identify the cause of pain, i.e. a bleed or not. Blood and bone can be differentiated in the image; any thickening of the joint, changes in the bone, cartilage, and the state of the synovium can all be identified; and what can be seen then graded from 0 (normal) to grade 1. 2. etc.

Ultrasound is a sensitive tool in detecting joint changes relating to subclinical disease alongside joint score measurement. The risk of bleeding can also be apparent from ultrasound imaging.

Ultrasound is of high educational value to the patient as the practitioner can talk through with the patient what they are seeing.

DR MARK HORSLEY: SURGICAL OPTIONS

BY NICKY HOLLINGS

Surgery is usually the end stage of joint management for haemophilia patients. The most common interventions are elbow replacement, ankle fusion, and knee replacement.

Elbow replacement

Elbow replacement can be difficult due to preoperative stiffness. The surgery increases the range of elbow movement, but not back to normal, and results in good pain relief at the joint. Replacing the elbow means that it is no longer a weight bearing joint.

Ankle fusion

This surgery replaces a stiff painful joint with a stiff painless one. Following the surgery the patient will have 12 weeks in a cast, then eight weeks where the ankle will be non-weight bearing.

Ankle arthritis options:

- Aethroplasty: Young, early failure
- Arthrodesis: Preoperative stiffness, difficult, non-weight-bearing for 8 weeks due to upper limb problems

Knee replacement

Also known as knee arthroplasty. As of 2008 in Australia there had been 35 knee replacements in 26 patients in over a 21 year period. The average age at replacement was 34 years old.

This surgery results in a long bed stay due to requirement for post-surgical factor treatment. The procedure provides good pain relief, but it will result in some post-operative stiffness.

Gene Therapy Demystified

BY SUE ELLIS

Dr Julie Curtin presented on gene therapy and non-factor treatment. She spoke about how non-factor replacement works, and how gene therapy fits into this context. She explained some of the jargon, talked about what gene therapy is, how it works, and what is achieved.

Haemophilia treatment via infusion has a higher risk of infection, so scientists have been looking at different ways to administer medication, for example subcutaneous, oral.

There are a number of novel treatments currently available, or in clinical trials:

EMICIZUMAB

Emicizumab is a nonfactor antibody, which is currently available as Hemlibra. It binds to FIX and FX, and is effective in haemophilia A including where there are inhibitors. Initially seen as a treatment for patients with inhibitors, there is now evidence that non-inhibitor patients will benefit too. See The New England Journal of Medicine: "Emicizumab prophylaxis in patients who have haemophilia without inhibitors".

In the Haven trials there was incidence of thromboembolism. There was one fatality related to bleeding and other factors, but not related to Emicizumab specifically.

Emicizumab is currently undergoing phase 3 trials for haemophilia A, non-inhibitors.

CONCIZUMAB

This is a Novo Nordisk product, currently undergoing phase 2 trials.

FITUSIRAN

This is a Sanofi product. It works by suppressing levels of antithrombin, resulting in a significant decrease in bleeding.

It is currently in clinical trials. There has been one death of a patient on the trial, determined unrelated to the medication, so the trial was on hold for a while, but has just started up again.

VIRAL VECTORS

Gene therapy is the art of turning infectious agents into vehicles of therapeutic medicines – replicating DNA and vector DNA. That is, modifying a known adeno-associated virus (AAV) to carry beneficial agents.

There have been a number of articles and much published research over the years on gene therapy. Current phase 3 observational trials. New AAV types will get better, but the biggest challenge is still the immunological impact on success. Not suitable for babies or young children, because their liver is still growing.

How long can gene therapy treatment last? That is still the big unknown. FVIII levels about 3 years post clinical trial may have stabilised, but there is a wide variation of levels across the cohort. There is still a lot to learn.

From Girls to Women

BY LYNNE CAMPBELL

This session was in four parts.

First, Shauna, a young woman with vWD who had been a ballet dancer, outlined her personal story. She had required factor on demand until age 24 then went onto prophylaxis after an accident. Treatment via factor was on her own terms so she had no compliance issues.

Education was invaluable to her, she was able to self-advocate, communication with her mother and the treaters was open, and no topic was taboo.

She found that to keep active prevented serious bleeding.

She was very grateful for advances in medicine

DR JENNY CURNOW: FEMALE FACTORS: ISSUES FOR DIFFERENT LIFE STAGES AND HOW TO HANDLE THEM

In her work as Director of Clinical Haematology Dr Curnow works to address the issues of:

- Heavy menstrual bleeding
- Iron deficiency anaemia
- Genetic testing
- Pregnancy and delivery
- Surgery and procedures.

She investigates these topics across the life stages, addressing the full range of bleeding disorders affecting women, where women bleed for longer than is normal.

- vWD is diagnosed 3:1 in women over men because of the problems women with vWD experience.
- A clotting level below 49% can be clinically described as Haemophilia.
- Only 5–10% of women seek medical attention for Heavy Menstrual Bleeding (HMB). There is high reporting of HMB from women with vWD.

Diagnosis of a bleeding disorder requires laboratory testing. It is easy to get false positive results as clotting factor levels can be influenced by triggers such as stress, exercise, hormonal changes, inflammation, and age. Platelet function testing is affected by medications, ginger, garlic, chilli, gingko biloba, etc.

Treatment for women with HMB is individualised to the age and stage of the patient, and can be via hormonal treatment, non-hormonal treatment, or via surgery. The Mirena IUD takes several weeks to work but provides very useful hormonal intervention, and can be used in combination with a non-hormonal treatment such as tranexamic acid. The point was made that many of the gynaecological issues faced by women with HMB are not necessarily the result of an inherited bleeding disorder.

Iron deficiency anaemia is a problematic adjunct to HBM. Prevention of bleeding is key to prevention of anaemia and an increase in iron intake is the primary treatment.

A lack of vitamin C can promote bleeding and anti-inflammatories promote bleeding.

Where a woman is known to be a high obstetric risk, it is imperative that the Haemophilia Treatment Centre is involved with monitoring factor levels, determining the nature of delivery of the baby, and post-partum monitoring should replacement therapy be required.

As women get older, there may be an increased need for surgery and procedures. It will be necessary to address bleeding risk in situations such as:

- Osteoarthritis: pain relief, surgery
- Cardiovascular disease: antiplatelet medications
- Screening for bowel cancer: preparation for endoscopy, colonoscopy
- Breast screening: preparation for breast biopsy
- Vaginal bleeding.

Important tips:

- Know your bleeding disorder and factor levels
- Carry a card or wear an alert specifying diagnosis, treatment, contact
- Before you have ANY surgery or procedures, no matter how minor, tell your Doctor, dentist, or surgeon about your bleeding disorder, and ask them to contact your HTC
- Contact your HTC Dr/Nurse to discuss whether medical management is needed to prevent bleeding and where the procedure can take place
- Before taking any new medicines, vitamins, supplements, or herbal remedies – check whether they are safe for people with bleeding disorders.

DR KIM MATHEWS: THE GYNAECOLOGIST AND PATIENTS WITH BLEEDING DISORDERS

Dr Matthews helps young women deal with contraception and fertility issues in their transition to adulthood. She aims to promote normal menstrual function, discuss sexual function, promote safe sex, treat gynaecological issues, and consider fertility options with patients against a backdrop of chronic illness.

There was some overlap with the first speaker about inheritance and HMB. Her focus was more on pain management, avoiding NSAIDs, as well as hormonal and non-hormonal interventions. She referred to the difficulties young women often experience when menstruation begins whether or not they have a known bleeding disorder.

In her discussion of PGD/IVF she emphasised the importance of avoiding unplanned pregnancies, the need to be monitored by a fertility specialist and the wisdom of single embryo transfer only in PGD to reduce bleeding risk and stress on the body.

Summary:

- Good to be aware of your individual circumstances
- Discuss with your team what is safe for you and what is not
- Seek appropriate contraceptive advice
- Plan for pregnancy after ensuring it is safe to do so
- Genetic health of the offspring should be considered
- Multidisciplinary approach important.

JOANNA MCCOSKER – NURSE PRACTITIONER: CARRIERS: WHAT DO PARENTS, YOUNG GIRLS, AND WOMEN NEED TO KNOW?

For every one male with haemophilia there are up to five female carriers of the haemophilia gene. Although we are moving away from the term carrier, in the case of haemophilia a "carrier" can also be "symptomatic" for the condition so in the case of haemophilia, women can bleed too. Women have many haemostatic changes in the course of their lifetime, with menstruation and childbirth being the most extreme, and unique to women.

An obligate carrier can be identified as follows:

- All daughters of a male with haemophilia
- Mothers of one son with haemophilia, and
  - who have at least one other family member with haemophilia, or
  - who have a family member who is a known carrier
- Mothers of two or more sons with haemophilia.

Over half of all carriers do not realise they have the gene for haemophilia. A delayed diagnosis of status is a barrier to appropriate management and timely treatment for those women who have the haemophilia gene. Risk factors and complications include bleeding, impaired quality of life, bleeding risk to the pregnant woman and baby and possible complications during pregnancy and delivery.

Joanna promoted the online Self-Administered Bleeding Assessment Tool (Self-BAT) developed by Dr Paula James, which is freely accessible on https://letstalkperiod.ca/

She also emphasised the need to develop, update, and record family history from a young age in order for women to be informed and manage their bleeding and reproductive risk.

Once the young woman is transitioning from school age to early adolescence and beyond to adulthood, it is important for her to link in to the HTC. Low iron levels may become an issue. To reinforce knowledge and promote informed decision-making is crucial. During the early reproductive years the young woman may seek to access genetic testing and require help with decision-making around reproduction. She may need a referral to the gynaecologist, genetic counsellor, or psychologist to plan her pregnancy, work through her reproductive options, and safely manage her pregnancy and delivery.

Self-advocacy

BY NICKY HOLLINGS

This session took the form of an interactive workshop: successful self-advocacy; identifying relevant skills and resources; barriers and overcoming them. The session was facilitated by Loretta Riley with Dr Lianne Khoo, Nicoletta Crollini and Jane Portnoy.

The sessions that I sat in on examined two questions and participants offered their thoughts and snippets of their experience:

1. What makes self-advocacy difficult?
    - Some patients struggle to speak openly with a specialist, for example power imbalance in relationship.
    - Some clinicians tell patient what is best for them and the patient lacks confidence to offer their viewpoint.
    - Fear – which might derive from a prior bad experience.
    - Not sure how to get my point across.
    - Some of the older specialists are difficult to engage with.
    - Not wanting to be a nuisance.
    - Lack of self-esteem and confidence in the hospital setting.
    - If self-advocacy is difficult for you then be prepared to have someone assist you. Make sure you explain the issues you have beforehand. If for example medication means that you feel you are in a brain fog, then important someone can advocate on your behalf.
    - Understand your strengths and weaknesses and when you need help.
2. Describe when self-advocacy worked for you:
    - Maintain eye contact with those you are talking to.
    - Understand the perception of what you think you are saying and what is being heard by the other party.
    - Consider carefully the way that we speak, i.e. take the emotion out of it.
    - Be prepared – jot down bullet points. Adopt an analytical approach.
    - Do a sanity check – check in with a friend or your physio etc. to see if your viewpoint seems both valid and comprehensible.
    - Be assertive but remain objective and courteous. Important to try and retain a working relationship.
    - Be purposeful – do you need more pain relief, care etc. provide the relevant information.
    - Calling E.D. ahead of time if this is possible. This allows for the haematologist to be called ready for when you get there. Have a plan for emergencies.
    - Ensure that you understand your rights are well informed and have knowledge of options about.
    - Understand your condition and the roles of the members of the HTC team.
    - Talk to others with similar experiences and try to be involved in and help maintain a support group.

WELCOME TO THE WORLD CAFÉ!

BY AMBER MAIHI

In this interactive session, Loretta Riley had us move between four tables that were set up as stations and answer questions about some of the barriers and struggles people with bleeding disorders face when trying to advocate for themselves. On each table sat one of the speakers who helped to facilitate the discussions. Each switch allowed all attendees to hear new perspectives and opinions. The group was diverse; a mix of psychosocial, clinical, and patients themselves.

The Questions:

- Describe a moment when self-advocacy worked well for you. What skills did you use to advocate for yourself?
- What makes self-advocacy difficult, and in what ways could these be overcome?

The response from a psychosocial view was that self-advocacy is very important and needs to be encouraged more from a younger age. Building the self-esteem of a child with a bleeding disorder will ensure that they are confident to give their opinions and feelings when it comes to their own condition. Barriers include PTSD, fear of exclusion, and lack of communication skills. This showed through most during the transition period from paediatric to adult care, as the centres change and are not familiar anymore.

Some of the proven ways to overcome these are; speaking to someone you trust about how you're feeling, and reaching out to a social/outreach worker for added support until you feel you're able to keep going independently.

For a person with a bleeding disorder, the key barriers to self-advocacy were clinical knowledge, being told that this is professional advice and they need to take it made them feel like they couldn't trust their own instincts; trying to communicate and not being heard and/or ignored; and the frustration of trying repeatedly with no positive outcomes. Transitioning to an adult treatment centre can be quite a daunting experience. Not seeing the same specialists and nurses you have grown up with can cause anxiety and stop you from wanting to speak up.

A few methods that worked well for patients practicing self-advocacy are seeking a second opinion of a professional that you know and trust, looking back at all the obstacles you have already beaten to empower yourself to carry on trying, and to reach out to your support networks.

Youth

WHAT'S THE RISK?

BY NICKY HOLLINGS

This was a panel discussion exploring different scenarios, with audience input (Q&A style). There were five scenarios put to the panel, and to the audience. The scenarios covered personal decision-making around visiting HTC for a bleed review, a gym/sport-related injury; travel; disclosure – in sport, in relationships; work, and the challenges of becoming an adult.

The panel included:

- Young people with bleeding disorders ¬
  - Alan and Sabrina
- Older person with haemophilia and parent ¬
  - Paul and Shane
- Health professionals
  - Greig Blamey, Physiotherapist
  - Jane Portnoy, Psychosocial worker
  - Steve Matthews, Haemophilia Nurse

SCENARIO 1

You have had an injury at sport last week and have been doing your rehab plan as recommended by your haemophilia physio, but your team is really short next weekend and they are desperate for you to play.

Your physio has said to take it easy for 2 weeks and it will be 10 days. You think that's close enough to play next weekend.

Audience: Medium risk.

Panel: Low risk

SCENARIO 2

You are travelling overseas and you haven't talked to the HTC. You know that they are busy and won't be happy that you have left things to the last minute.

You are wondering if you should just pack the treatment product you have in your fridge and hope for the best.

Audience: High risk

Panel: Low risk

This can lead to high risk very quickly depending on how isolated you are, or you could pick up treatment from a service near where your event is, so can be very dependent, though always take a letter when going overseas, and have insurance.

SCENARIO 3

You have started at university and met some new people. You really enjoy hanging out with them, and you have a lot of shared interests. You can see some good friendships coming out of this.

Should you tell them about your bleeding disorder?

Audience: Low risk

Panel: Low risk

There was discussion around people feeling vulnerable at this time. Disclosure would lead to understanding how you can cope with rejection, if this comes up.

SCENARIO 4

You play sport regularly and have a treatment plan for injuries. You are pretty used to managing things, and usually this works well for you. You have had an injury that seems a little bit different to the usual, but you are not sure.

You are pretty busy with work and study and think you will see how things go in the next couple of days before you contact your HTC.

Audience: Low risk

Panel: Low risk

This too could turn to high because it seems different to the usual. Patients usually know what's right, but sometimes just a phone call to the HTC could help with better treatment.

SCENARIO 5

You have travelled interstate for a music festival and have had a bleed, you have used more treatment product that you expected, and you don't think you will have enough to cover for your treatment plan while you are away, but it's only a few days.

You are wondering if you should ration what you have and just have a bit less for the rest of the week.

Audience: High risk

Panel: Medium risk

It's important to recognise that this too could go quickly to high due to being active at the concert, which could lead to more bleeds.

Loretta Riley, Anne Finlayson, and Dr Jane Mason: Queensland Haemophilia Centre's Experience of Telehealth – Engaging with the Rural, Remote and Regional Inherited Bleeding Disorders Community

Queensland is the second largest Australian state with the most decentralised population. However, most specialist services are in the South East including the Queensland Haemophilia Centre. As an addition to outreach visits, both the Queensland Children's Hospital team and the Royal Brisbane and Women's Hospital team have expanded their services to include telehealth clinics in order to improve access to appointments with the Centres. Both teams will share their experiences; including the challenges and benefits of telehealth.

Why use the telehealth service? It increases accessibility to health care service, minimises the cost of travel to participate and working around family commitments. The patient can undertake the appointment from home, hospital site or an office. It has been found to improve bleeding disorder education, with all disciplines represented at the meeting. Individual appointments can still be made, for example physiotherapy appointments.

The benefits of this service include the Aixip app, used to facilitate the meetings, which is free to participants. The system allows for a virtual waiting room.

The service operates out of seven cities. The link can be by video, text, or email depending on the technology available to the patient. There are always two options available to patients, a face-to-face option or an ad hoc clinic. Generally, email is used to convey information about how the appointment will be conducted and there are occasions where face-to-face appointments will be necessary.

New Opportunities or is the Status Quo Good Enough?

BY SUE ELLIS

Michael Stone; an expert on evaluation of new therapies, payment models, and reducing barriers to access; talked about the need to have standardised outcome measures in place that are developed and designed with all stakeholders.

What I liked most from this presentation was the emphasis on consumer engagement and I include the bullets from the concluding slide of Michael's presentation that shows this:

- The higher the expectation for new treatment, the more critical to choose the proper measures.
- Harmonising measures across studies is the single way to support informed decision-making.
- Looking forward is important, but not trashing the past is as critical.
- The contribution of patients in identifying and generating appropriate outcomes is critical.

WFH eLearning platform

One stop for easy access to all resources on inherited bleeding disorders: The eLearning Platform is your one stop for easy access to all WFH educational materials. You will find everything you need on hemophilia and other inherited bleeding disorders, from a quick introduction to in-depth guides and articles.

Home to foundational WFH resources such as the Treatment Guidelines and Lab Manual it features over 650 other resources including interactive learning modules, educational videos and games, webcasts, and online tools for healthcare professionals.

Official WFH translations in Spanish, French, Arabic, Russian, Simplified Chinese, Portuguese, and Japanese, and NMO translations into another 18 local languages are freely available to all!

For all your advocacy, medical education, and learning needs, head on over to the WFH eLearning Platform.

Treatment Transition

When you take a medication regularly, changing to a new one can cause worry and insecurity. With the recent changes to Pharmac's preferred supplier of treatment for HFNZ members, we wanted to hear how the transition had worked out. Find out how Te Whainoa Te Wiata and an anonymous HFNZ mother felt about their changes.

CHANGE OVER TO NEW TREATMENT

BY TE WHAINOA TE WIATA

Kei te hunga pānui, tēnā koutou...

Advancements in haemophilia care over the last decade have jumped leaps and bounds from the days of my upbringing. A 7-day holiday often meant packing copious amounts of ice for the travel, and, on most occasions, a mini fridge to store the treatment. These days I have been fortunate to be able to pack what I need in my backpack, and the news of longer acting treatment is yet another step in helping us as 'people with haemophilia' (PWH) live our lives. It is always important to remember that having treatment is something to be grateful for, but it is equally as important to remember that everyone is different, so too are their experiences and what follows are some of mine.

I will be honest; the idea of reducing the amount of infusions came as very welcome news. Going from daily injections to twice or three times weekly would mean my veins get to have a rest, travel would be easier, and life overall can be a little smoother. Yet, I did not think I would encounter the mental adjustment that I have undergone. My first week was an eye opener. Long story short, I had been on a daily dose of 1000 IU for 20 years and didn't realise how accustomed I was to the feeling of being covered (by factor) every time I left the house. So, I was a bit nervous to say the least and I am still adjusting to it.

Practically speaking, I am still getting used to not having gauze swabs in the package, which may not seem like much, but has proven to be a little bit of an obstacle especially when having a bleed. Also, the prep and wastage are a lot more than the package of the previous treatment product and this I have found a little frustrating. However, these are just minor adjustments; my bleeding is neither better nor worse, which highlights the need to be mindful of lifestyle regardless of the product. As it turns out, the 'Longer acting' part of it all doesn't really happen for my body and me, thus it's just like normal treatment.

Overall, the change-over for myself has been frustrating to say the least, but the Auckland treatment centre (and treaters) have been amazing, so too, has the support of our HFNZ community. To close, I cannot speak for others, though, I feel that there should be some kind of trial period with patients and treaters before a final decision about product is made, as the one-treatment fits all, in my recent experience, is not the ideal avenue for better care.

E mihi ana ki a koutou katoa

Te Whainoa Te Wiata

Treatment table at youth camp 2015

OUR EXPERIENCE OF CHANGING TO THE NEW LONG-ACTING FACTOR

BY ANONYMOUS

For me, the experience of changing factors triggered a mixture of emotions. Initially, I felt excitement at the prospect of a longer acting factor, less frequent treatment times, and hope for more autonomy for my son. To date, I am still treating him, due to his lack of confidence in treating independently. This has historically affected his ability to go to school camps independently, long-term stays with other family members, and or friends. Long acting factor would mean increased independence for us!

However, there is always some anxiety with changing factor, fear of inhibitors developing, factor not being as effective with risk of breakthrough bleeds, and of course the changes in logistics of physical treating. The day he changed to his new factor, I was informed that the 'long acting results' were not as 'promising' as previously hoped. This left me feeling apprehensive about changing. I was also aware that there is a new longer acting, subcutaneous treatment out there that has shown 'life changing' effects. I began to feel frustrated that this was not available in NZ, and we were being changed from a factor that has been effective in maintaining his haemophilia, to a possibly less effective treatment. Our son is very active, so getting his treatment regime right is essential.

He needed to change the factor product regardless, with funding for his current product ending, so we decided to try out the new 'longer acting factor'. His blood results came back disappointing with a 24 hour drop in his factor levels to 5%, and a 1% level four days following (previous factor levels never dropped below 5% between prophylaxis treatments). In consult with his haematologist, a three day treatment regime was initiated. I was impressed with the haematologist taking the time to return my call and talk with me for an hour after his working day, as we modified his treatment plan. His flexibility in adjusting the regime was also appreciated.

Although I was hopeful for a 4-5 day prophylaxis treatment regime, every third day has positively impacted our lives. It is less intrusive on him, and he has more freedom to go away for overnight stays. Understandably, I am still hesitant for him to go independently to school camps (over three days). We eventually got our heads around the new mixing regime (initially, mucking it up and experiencing a state of panic). We have had to use our old unused needles as they are smaller than the ones offered with the new product, and spare prep pads as these are not provided with the new pack (I knew they would come in handy one day).

We had a good discussion with haematologist at the HTC about longer acting factor verses shorter acting factor. In particular, the quick initial factor drop off with longer tail (LAF) verses slower half live but quicker drop to 1% (SAF) - and implications of both for an extremely active child. I did not feel pressured to shift to long acting, but knew he would have to change to another product in a couple of months, so it seemed worthwhile giving it a go.

We had no support from pharma in relation to product other than false info that it needed to be chilled. We did call the helpline when our initial mixing of factor went horribly wrong, but were met with an automated voice message stating that office hours were closed. With limited time to use factor before it expired, we problem solved and eventually managed to extract it from the glass bottle.

I continue to feel nervous about the effectiveness of the current treatment, with more bruising noticed. He has not had any breakthrough bleeds that we are aware of, but there is always the fear that micro bleeding may occur. There are days we forget to treat, with the inconsistent days each week, but mostly we are able to maintain the current regime.

I am forever grateful for fully funded factor in our country, but look forward to the day when all individuals with haemophilia can treat subcutaneously with longer acting factor.

New Ultrasound Scanning Brings Improvements for Haemophilia Care in New Zealand

BY CAT POLLARD, ADVANCED CLINICIAN PHYSIOTHERAPIST, AUCKLAND REGIONAL HAEMOPHILIA SERVICE

We are donning our x-ray vision goggles and looking deeper into joints than ever before. Well, not really but at the main Haemophilia Treatment Centres (Auckland, Wellington and Christchurch) the specialised haemophilia physiotherapists have been trained to use ultrasound imaging to more closely monitor joint health.

Over the next year we will be incorporating into our yearly assessments an imaging protocol called the Haemophilia Early Arthropathy Detection with Ultrasound (or HEAD-US for short). This means we will be using the ultrasound scanner to look at the elbows, knees and ankles. These are the joints most commonly prone to bleeding episodes. In order to detect and grade any changes to the synovium (which is the layer surrounding the joint which helps produce fluid to keep the joint lubricated); the cartilage (the protective coating covering the bone ends); and the bones.

As we know, even one bleed into a joint can cause subtle changes to the joint as a whole, and more than one bleed can substantially alter the synovium, cartilage and eventually the bone surface itself. When a bleed occurs, the blood that leaked into the joint is removed by the synovium but the process of this irritates and inflames it, causing it to grow. This then makes it easier to pinch the synovium when the joint is moving, triggering another bleed. This leads to a vicious cycle of more frequent bleeds and more damage and changes occurring. Clearly picking up any of these changes early and taking measures to stop this from happening will help to minimise this cycle and keep the joints as healthy as possible.

The HEAD-US protocol is being used in other countries as well as New Zealand with great success. A research study to look at the effectiveness of the HEAD-US protocol demonstrated that it was able to identify a greater proportion of joint abnormalities than physical assessment with our usual Haemophilia Joint Health score. However, this is not to say that the joint score is no longer useful as it is still able to identify other issues which would not be detectable with imaging alone.

In our short time using the ultrasound scanner in Auckland we have already found it invaluable to assist in guiding treatment and identifying issues early.

One such case involved a seven year old boy with severe haemophilia who had experienced three bleeds in the same ankle over a seven month period. On physical examination there was only a small amount of swelling to the ankle and a very slight reduction in range of movement compared to the other side. However, when we used the ultrasound to look deeper inside the joint we were able to identify significant changes to the synovium, cartilage damage and changes to the growth of the bones in his ankle joint (see images).

Image 1: Scanning plane

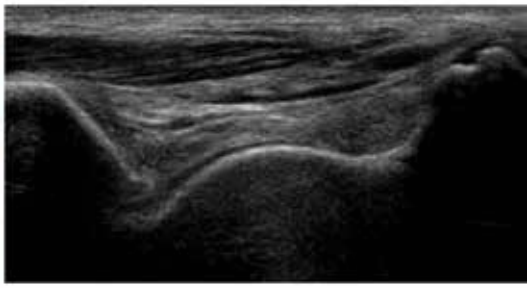
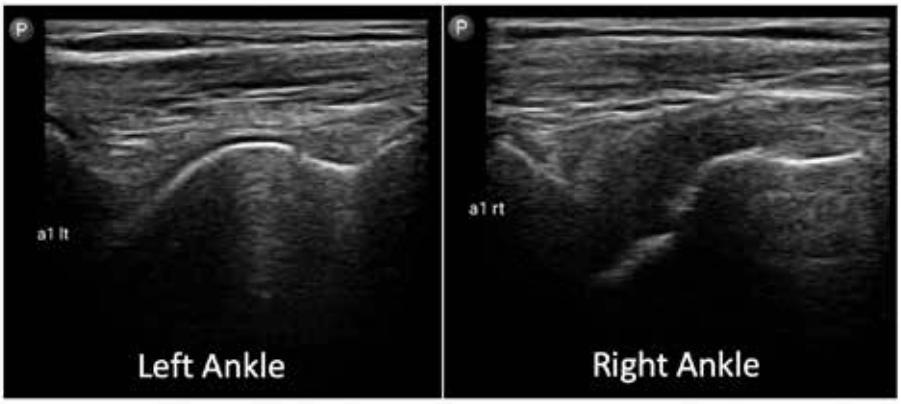
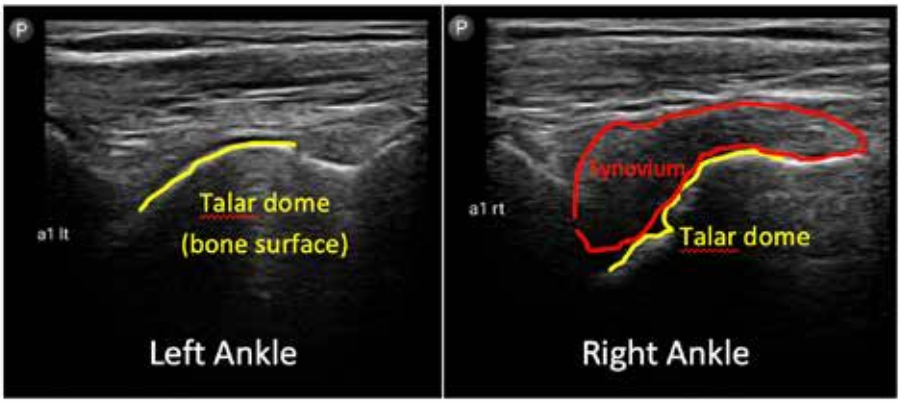


Image 2: Normal Anatomy seen in this plane



Images 3 and 4: Images of the ankles for Case study patient 1.



Port Hills Walkers

Well haemos, where have we been since we got our walks underway in late July?

Our first walk took us from the Huntsbury Carpark up to the Summit Road through the bush on the side of the road and down to the Sign of the Kiwi. The next took us from the end of Bowenvale Avenue up a track to the forest leading to Victoria Park, which in turn led us on to the Harry Ell track up to the Sign of the Kiwi (we like this place as a destination because we can sit down for a rest and have a cuppa!)

The third walk took us out on a loop walk from the Taylors Mistake carpark out to Godley Heads and back with magnificent views from the top in all directions. Our last walk from Elizabeth Park (adjacent to the Dog Park) and just below Victoria Park, again went up to the Sign of the Kiwi.

Remember, there are significant health benefits in hill walking, and for every kilo of excess weight we take off our joints, we lengthen their longevity. Regular walking of this type will also improve our aerobic fitness. Our sense of wellbeing when we are in the bush or stop to take in the wonderful views and the mateship being out there with others are other positive benefits.

Thanks for a great first year; we look forward to seeing you again soon. Please check out the selection of pics from the walks undertaken so far.

Ross and Kyle


SCHOOL

Regions and Groups

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

CENTRAL REPORT

BY BLAIR WIGHTMAN

2019 was another busy year for the central region, and it has been great to see some new faces at our events. We decided not to hold a Christmas event because the 60th celebrations were being held in Wellington in November. We also congratulate our committee secretary Ashley Fowlie on the birth of her son in September and look forward to welcoming him to our events!

Our Men's fishing charter was postponed due to weather for the fourth time in November and we're now booked for 16 February. We have a few spaces on the boat so please get in touch if you're interested.

Looking ahead to 2020, our central region Camp has been booked for the weekend of 28–30 August and we'll be holding it at El Rancho in Waikanae. This has previously proved to be a very popular venue with a great range of activities. Please mark it in your calendars.

NORTHERN REPORT

BY GREG JAMIESON

We had a wonderful Christmas catch up with around 60 of our Northern members at The Grounds in Henderson on 1 December. The kids were let loose on the playground and the adults had a chance to catch up and connect.

Our next summer event will be held at Parakia in February so please look out for the invite

Amber, our Outreach Worker, fits into our Northern family nicely, and is doing a great job in supporting our families. If you haven't meet Amber yet, please take the time to do so, as she is making a difference!

We did a planning session with a few of our members at The Grounds. We are looking at 3–4 events for next year including Rainbows End in Dec 2020, possibly a movie event/rock climbing, and a dinner outing. If you have any ideas on the events you would like to attend, please email Amber northern.outreach@haemophilia.org.nz

If you want to be a part of the committee, or would like to help organise or contribute to some of the events, please also email Amber at the above address.

PIRITOTO REPORT

BY ROSALIE REIRI

Kei aku rangatira, tēnā koutou katoa.

kāranga mai te reo o te pīpīwharauroa, e karanga ana ko te raumati, ko te hararei, te whakatā me te noho tahi ā-whānau.

Greetings to all, as the pīpīwharauroa calls, she calls the summer, the holidays, a break, and also time together with our families.

Firstly, on behalf of Piritoto, we wish to thank the organising team of HFNZ's 60th birthday for a fantastic night enjoyed by all those who attended. Thank you also for the tokotoko that was gifted to Piritoto, it was thoughtful and took us all by surprise. Ngā mihi.

Last year it was suggested that Piritoto should support the 60th birthday by performing a kapa haka set and so this year we did just that. We put together a 10-minute bracket of Waiata, with songs representing each region that we all come from, including some good old favourites, and songs of welcome. I wish to pay tribute to our whānau who supported this, who practised in their own time, and also those who have never experienced performing kapa haka before. Tēnā ka mihi atu ki a koutou.

What does 2020 entail? Wellness, wellbeing and whānau. We would like to welcome into our whānau Kyle and Amanda in the South Island. I also wish to acknowledge Kyle, who was recognised at the 60th for his inspirational story of health, fitness, and wellbeing. There are some ideas floating around getting our whānau walking and perhaps doing a collective effort towards the Auckland marathon in 2020.

I wish to acknowledge our kaumātua of Piritoto, those that were there in the early days like nanny Nel, and those who are forever guiding us, papa Pete and nanny Helen, and also our overseeing matriarchs Robyn and Patience. Aroha nui atu ki a koutou katoa.

We hope you had a wonderful break and that your Christmas was everything you needed.

Meri Kirihimete me te tau hou harikoa 2020!

SOUTHERN REPORT

BY SUSAN INWOOD

The Southern team have had a good year. We had our AGM on 22 September, with a new committee elected, and some extra members on the committee, which is great to see.

The 60th anniversary event was a great chance to catch up with our Haemophilia family, and it was wonderful to see so many Southern people there. The welcome reception at Parliament and gala dinner were a treat. The weather did not behave, but Wellington cannot help itself...

Kyle Cunningham and Ross Paterson, our Outreach Worker, have started a walking group for any members who want to get out into the fresh air and get some exercise with like-minded friends. By all accounts, this has been very popular. Check your emails for information on the next walk.

Regions and Groups (continued)

With Ross's help, we have organised a Fishing trip for Southern members in early 2020. We will have two trips: one from Moeraki in February, and one from Nelson in March. We have had a wonderful response, so get those fishing rods cleaned up!

Our Christmas event this year was held at Abberly Park. There were some great activities, and the weather played ball. It was great to see so many Southern members.

MIDLAND REPORT

BY TINEKE MAOATE

Hi to all our members, friends, and family of HFNZ. A big thank you to all the people that organised the amazing 60th celebrations. This was attended and enjoyed by many. It was great to catch up with friends that we haven't seen for a while. While we were in Wellington, I had the pleasure of hosting a morning tea at the Cable Car Eatery for the people and family members with von Willibrands and other bleeding disorders. This went really well and it was great to have a good turnout.

We have a new committee and are all excited to be working together for HFNZ Midland.

We had our Xmas event at Off Road NZ on the 8 December. Don't worry if you couldn't make it as we are planning a summer BBQ at Blue Lake in Rotorua. Hope to see everyone there.

Looking forward to 2020 and what it has in store for all of us.

Getting ready for the dance NFC 2019

THE YEAR AHEAD

March 13 & 14, 2020

- Inhibitors Workshop
  Auckland. Quality Inn Parnell

April 17, 2020

- Premier of Bombardier Blood Movie
  Christchurch. Venue to be advised

July 9–12, 2020

- Youth Camp
  Auckland. Venue to be advised

November 6–8, 2020

- Adult Weekened
  Christchurch. Venue to be advised

April 29 – May 2, 2021

- National Family Camp
  Kaiapoi, Blue Skies

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

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