Haemophilia Foundation of New Zealand Inc.
HFNZ

bloodline

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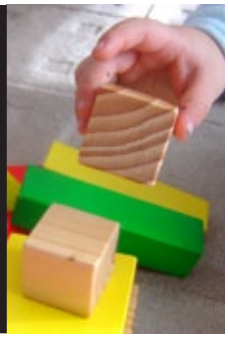

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The H Word

What a whirlwind the last few months have been. First and foremost was all the excitement of the 2014 WFH World Congress which took place in Melbourne 11-15 May. All the Kiwis certainly made their mark at Congress. It was an amazing experience to have a Congress so close by so that so many HFNZ members and haemophilia treaters could take part.

Thank you to everyone who fundraised through your MRGs for the last 4 years to enable members to attend! You can read all about the latest updates and experiences in our special Congress coverage.

On a sadder note, Belinda Burnett has moved on from her position as Chief Executive of HFNZ, after 10 years in the role . Thank you Belinda for your contribution to improving the lives of people with bleeding disorders over the years.

We anticipate appointing a new CEO shortly and look forward to introducing them to the community at the upcoming National Annual General Meeting taking place in Auckland on 13 September.

Deon York
HFNZ President

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www.facebook.com/haemophiliaNZ

Masters Group (request to join)
www.facebook.com/groups/hfnzmasters

Farewell Belinda!

As many of you are aware, Belinda Burnett has been our Chief Executive for over 10 years. During that time HFNZ has undergone many changes and faced many challenges. Belinda has been the constant during all these years and has served our community with dedication and determination.

In March Belinda decided to take up a new opportunity as the Chief Executive of Cystic Fibrosis New Zealand. Her last day at HFNZ was 17 April, World Haemophilia Day. Staff in Christchurch marked the occasion with a special lunch and an office 'open-house' where members and associates were invited to come wish Belinda well or share a memory.

Belinda has been key part of the Foundation for so long and there are many memories indeed.

Belinda was appointed to the role in 2003, when it became clear that the volume of activity that HFNZ wanted to achieve had begun to exceed what could reasonably be coped with by a volunteer National Council. The position was labelled "chief executive officer" because HFNZ wanted someone who, in addition to managing staff, would be able to open more doors in the business world and in government, and give the Foundation an added degree of professionalism.

Belinda applied for the role because she felt her background in marketing would complement KiwiFirst when it came to fundraising for HFNZ. The mother of a person with haemophilia and a former Southern branch chairperson and delegate to the National Council, she also understood both living with haemophilia and the workings of the Foundation.

Her first focus as CEO was to develop personnel procedures and policies. Focusing on 'working smarter', she and the Council began examining how to make the most of the precious "H" dollars, finding new funding sources and helping Outreach contact as many members as possible – especially those in rural areas.

The appointment of a CEO was a huge leap forward for HFNZ as the National Council was able to begin handing over some of the long-term planning issues and day-to-day management to Belinda, allowing council to concentrate on policy and people.

As Burnett grew into the role, she managed to create lasting and important relationships with a number of companies and organisations, raising HFNZ's profile in New Zealand and internationally. She also managed to secure a number of new funds and worked hard at the delicate art of juggling all the demands, and worries that come with the job. In addition, she has proved a great manager to the staff, ensuring their concerns are listened to and encouraging them to use and expand their talents for the benefit of the Foundation and its members.

Belinda has played key roles in many of HFNZ's achievements in recent years, such as in the crusade to achieve the Treatment and Welfare Package for people affected by hepatitis C through contaminated blood, especially in getting the experts from Ireland to come to New Zealand and help win the fight. She has been a champion of promoting the issues of women with bleeding disorders and the fantastic workshops run by HFNZ to other haemophilia organisations around the world and ushering in the professionalism of the Foundation today.

Some of the accomplishments she is proudest of involve the merger of the finances of the four branches into one entity in 2007 which required her to quickly become an expert in the Charities Act and moving the organisation from a management committee into a Governance and Management style of operating. In her time as CEO, Belinda also oversaw the funding received as grants from pharmaceutical companies increase several times over and built a close-rapport with the Ministry of Health.

2012 National Council

"My role at HFNZ has certainly been the most significant job of my career to date," reflects Belinda. "The challenge of working for Cystic Fibrosis and learning about a new condition and all that entails will in no way change the depth of connection I have at HFNZ. The many years of involvement I have had with HFNZ is an experience I would not change for the world (and of course - I'm still a carrier!)".

"My final words on my time at HFNZ are that the members are so lucky to have the staff they have. It's been a privilege and a pleasure working alongside people that are passionate, resilient and committed to the cause. I hope everyone realises just how much additional work the staff do; always going the extra mile and being totally committed to haemophilia".

Belinda concludes, "And to all the wonderful, resilient and inspirational people with a bleeding disorder; I can't begin to tell you how much I have learned from you all. You have taught me to be thankful for all I have in life and for the many blessings I have".

HFNZ staff and the National Council are all working to ensure her departure has minimal disruption to members. Recruitment of a new chief executive is well underway and an announcement will be made as soon as possible.

While we may be losing Belinda as an employee of HFNZ, she is still a member first and foremost and will always be a part of the wider haemophilia family. We wish her the very best for her new position.

Belinda with the Christchurch-based HFNZ staff at her farewell.

Practice Preventative Health

Men's Health Week recently took place from the 9-15 June 2014. The event provides a great opportunity to think about your current health and take stock of how well you are protecting your health.

Health is about a lot more than haemophilia. In general, men tend under appreciate the importance of health prevention strategies. Although people with haemophilia sofemtimes expect all their health care needs will be met by a routine visit to their haemophilia treatment centre (HTC), HTCs can lack the expertise to deal with general age-related illnesses and conditions. Therefore it is important to also have a GP that knows you well and can help treat non-bleeding disorder related conditions.

Practice Preventative Health

Even taking simple steps to protect your health can have a big difference in the long run. See the list of simple steps you can start taking today to improve your health.

Medical research has come a long way, and as a result, people live much longer than they used to. To help you live the healthiest life possible, people with bleeding disorders need to start planning early to age positively. Older age is a time to live well, be happy and as healthy as possible. Most people also aim to be independent and in their own homes for as long as possible.

Learning about and practicing preventive healthcare, i.e., maintaining your body and good health throughout your entire lifetime, is probably the best method to prevent disease from happening in the first place. This means acting now – no matter how young you feel!

When you are sick, you have no choice but to think about your health; it's right there in your face – you feel awful. Preventive health care must be planned and executed ahead of time, even when illness is absent. This means building healthy habits even when it's not convenient and believing that maintaining a healthy lifestyle is important enough to make a few sacrifices. Eating right, getting exercise and avoiding or reducing damaging substances like tobacco, alcohol and excessive amounts of sugar and salt are crucial, as is getting enough sleep each night. Healthy habits are an "automatic" defence against most illnesses and can provide us a longer, healthier - and hopefully happier life.

Another aspect of preventive healthcare is finding and treating disease as soon as possible. Bleeding disorders aside, some sort of illness hits everyone eventually, but many, many diseases – when caught early – can be nipped in the bud and full health returned quickly. People with bleeding disorders are not immune to major health risks such as diabetes, cancer or depression. So it is important to become educated about illness and their symptoms, and what it takes to maintain good health overall.

Moreover, it is also important to visit your GP on a regular schedule – at least once a year or possibly more often as you get older. This can help you stay healthy and catch any disease early. If detected soon enough, a full cure is much more likely. It is also much more unlikely that most disease will become serious enough to negatively affect your lifestyle with debilitating symptoms or even loss of life.

The health checklist on the next page can be used to help you think about health issues that may be affecting you now and in the future. It is only meant as a guideline. Tests and screening procedures that you may need will depend upon your individual risks, your age, and your family history. Talk to your GP and find out what is needed to maintain your health.

Simple steps to protect your health

1. Visit a GP. One of the easiest ways to take care of your health is having a check-up once a year. By monitoring your health and being aware of any illnesses in your family history, you will be more likely to catch any health issues early. Detection is in your hands.

2. Measure your pressure. Blood pressure can be a key indicator for a range of illnesses, such as stroke.

3. Exercise regularly. The benefits of regular exercise can be huge for physical, mental and sexual health. Take control of your health with 30 minutes of daily exercise.

4. Eat healthy. Eating well is important for both mental and physical health. You need to know what foods to eat and what foods to avoid in order to minimise health risks.

5. Healthy thinking. Recognising the symptoms of depression in yourself and others can be the first step to beating it. Remember there are services out there to help you or you can chat with your GP.

6. Stop smoking. It's the only healthy option.

7. Maintain healthy relationships. Close relationships with family and friends are the most important source of love, support and understanding. One of the best things for men to do is make sure they have close mates who they feel comfortable enough to talk to about anything.

Men's Health Checklist*

*** Adapted from 'Men's Health Check', Northern Sydney Central Coast Health, 2005**

18-39 Years	40-49 Years	50-64 Years	65 Years and older
Some of the big picture issues that you need to watch out for … • Early stage heart disease, high blood pressure & stroke • Testicular cancer • Injuries • Relationship difficulties • Drug & Alcohol issues • Overweight • Skin Cancers such as Melanoma • Mental health & wellbeing	Some of the big picture issues that you need to watch out for … • Heart disease, high blood pressure & stroke • Diabetes • Drug & Alcohol issues • Overweight • Respiratory illnesses & lung cancer • Skin cancers such as Melanoma • Mental health & wellbeing • Erectile difficulties	Some of the big picture issues that you need to watch out for … • Heart disease, high blood pressure & stroke • Diabetes • Drug & Alcohol issues • Overweight • Respiratory illnesses & lung cancer • Bowel, prostate & skin cancers • Mental health & wellbeing • Erectile difficulties	Some of the big picture issues that you need to watch out for … • Heart disease, high blood pressure & stroke • Diabetes • Drug & Alcohol issues • Overweight • Respiratory illnesses & lung cancer • Bowel, prostate & skin cancers • Mental health & wellbeing • Erectile difficulties
Your health check might include: • Physical exam by your GP every two years, including blood pressure checks, height and weight. • Cholesterol (test for cardiovascular disease) • Baseline reading in your 20s with follow-up tests as recommended by your GP. • Blood glucose (to test for diabetes) if you have high blood pressure and high cholesterol. • Skin exam every year (screening for skin cancer) especially in men who have a high exposure to the sun and other risk factors. • Dental exam and cleaning every year. • Self-examination of testicles for lumps. • Immunisation review every 10 years.	Your health check might include: • Physical exam by your GP every year, including blood pressure checks, height and weight. • Regular cholesterol screening (test for cardiovascular disease) at least every 5 years or at your health care provider's discretion. • Blood glucose (to test for diabetes) if you have high blood pressure and high cholesterol. • Skin exam every year (screening for skin cancer) especially in men who have a high exposure to the sun and other risk factors. • Dental exam and cleaning every year. • Self-examination of testicles for lumps. • Immunisation review every 10 years	Your health check might include: • Physical exam by your GP every year including blood pressure checks, height and weight. • Regular cholesterol tests (for cardiovascular disease) at least every 5 years or at your GPs advice. • ECG (heart trace) every 3 – 5 years as recommended by your GP. • Test for diabetes (fasting blood sugar) every 3 years. • Screening for bowel cancer every 2 years including colonoscopy every three to four years as recommended by your GP. • Prostate checks annually. To test for prostate cancer as recommended by your GP. • Skin exam annually as part of the physical exam (screening for skin cancer). • Bone density test as recommended by the GP. • Eye vision test annually. • Dental exam and cleaning every year. • Immunisation review every 10 years.	Your health check might include: • Physical exam by your GP every year including blood pressure checks, height and weight. • Cholesterol test every year at your health care providers' discretion (for cardiovascular disease). • ECG (heart trace) every • 3 – 5 years or as recommended by your GP. • Test for diabetes (fasting blood sugar) every 3 years. • Prostate checks annually. To test for prostate cancer as recommended by your GP. • Screening for bowel cancer every 2 years including colonoscopy every three to four years as recommended by your GP. • Skin exam annually as part of the physical exam (screening for skin cancer). • Bone density test as recommended by the GP. • Flu shot & Eye vision test annually. • Hearing screen every 2 years. • Pneumonia vaccination at 65 then as recommended by GP. • Dental exam and cleaning every year.

You might want to visit the Men's Health Week website ***(http://menshealthweek.co.nz/)*** to fill out the 'What's Your Score' survey and find out your annual health score. The website also gives you the chance to meet some of the high profile New Zealand spokesmen who have taken control of their own health and hear their stories.

Don't wait until symptoms are present – practice preventative health. Preventive healthcare should be considered an investment in your future. Every positive change is a step towards better and longer lasting health and happiness.

In memorial: DJ Elisa

Dominic Jnr (DJ) Elisa was a larger than life character with his big smile, cheeky giggle, playful manner and kind heart. As a boy with severe haemophilia with inhibitors he constantly showed great courage, strength and grace to overcome many adversities. He was a true inspiration and touched the lives of many people. Sadly, DJ passed away from complications following a head bleed in early June. He was 7 years old.

DJ's Parents, Cecelia and Dominic come from a close knit family from the Cook Islands. Cecelia was first introduced to HFNZ when she helped out at a New Families Camp many years ago through a family connection. DJ was born in New Zealand in 2007 and spent his first few months in the Cook Islands until a serious bleed led to the family settling in New Zealand and DJ being diagnosed with haemophilia.

The Northern members of HFNZ have always loved having the Elisa family along to their events, especially the regional summer camp they attended each year. DJ had so much fun playing together with other children with bleeding disorders. He will be truly missed by those children and adults alike, but will always be in their hearts, minds and prayers.

Many Northern families have said that it has been a great blessing getting to know the Elisa family, who have encouraged so many of them on their own haemophilia journeys more then they will ever know.

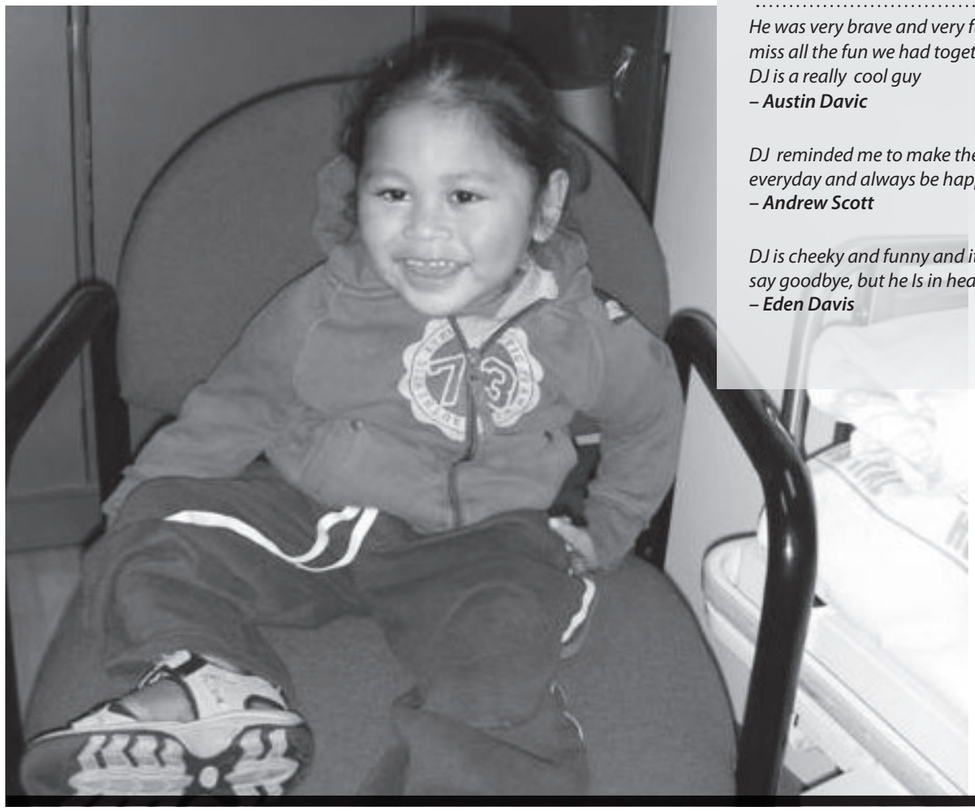
Cecelia and Dominic were fabulous loving and dedicated parents who let DJ participate and live his life to the full, yet were always there to look after him and protect him when needed.

DJ's medical team also cared deeply for DJ. "DJ always such a joy when he came to the HTC", remembers Mary Brasser. "He came in with that big bright beautiful smile, even when he knew he was about to get a needle and receive treatment – he was always so positive and brave."

The family held a beautiful memorial service that was attended by many Northern families and medical staff. The family is travelling back to the Cook Islands to lay DJ to rest.

DJ adored music and he was surrounded by guitars and beautiful singing in his hospital room. And though DJs song is now over, his melody will always linger on – and we will always remember DJ Elisa.

On behalf of HFNZ, from all the staff and all the membership, we extend Dominic and Cecelia our deepest sympathies. Please know that we are all here for you and always will be. Everyone is sending their strength and blessings from around the country.



Words from some of DJ's friends:

He was very brave and very funny and I will miss all the fun we had together at camp. DJ is a really cool guy
– Austin Davic

DJ reminded me to make the most of everyday and always be happy
– Andrew Scott

DJ is cheeky and funny and it is very sad to say goodbye, but he Is in heaven
– Eden Davis

Buddy Awards Coming to Australia and NZ

There were many exciting announcements at this year's World Federation of Haemophilia Congress, which took place over 11-15th May at Melbourne Convention Centre. From new research advancements through to investment announcements there were many things to celebrate. Not to be outdone in terms of important initiatives, the Buddy Awards were launched over breakfast to an enthusiastic audience hosted by Sharon Caris, Executive Director of HFA, and Colleen McKay, Manager of Outreach Services HFNZ.

The Buddy Awards recognise the significant medical, emotional and practical support provided by family, friends, healthcare professionals, teachers and others, to people living with bleeding disorders. The Awards, which take place annually in the UK, celebrate the invaluable contribution these caring people make while also drawing public attention to the challenges of living with a bleeding disorder. "The lack of public awareness of bleeding disorders means that the important role of families and friends often goes unnoticed" said Colleen McKay.

Sam Bradley from New Zealand marked the official opening by submitting the first nomination, for his wife, Donna, to recognise the continued support she provides. Sam explained "Donna has always been right by my side and has supported me for over 14 years". His speech left most with a tear in their eye and was closed with an impromptu song from the Kiwi members of the audience, a very soulful rendition of 'You Are My Sunshine'.

The award programme is sponsored by Novo Nordisk. Global Product Manager, Dr Peter Ramge, presented a donation to HFA and HFNZ to help promote awareness of the Buddy Awards during the breakfast.

HFNZ plan to roll out the Buddy Awards in New Zealand in early 2015. Look out for more information in the December issue of Bloodline.

Choosing a day care centre

By Sarah Elliott, Northern Haemophilia Outreach Worker, and a mother of a 3-year old with severe haemophilia

Starting childcare is a major step for families living with a bleeding disorder. For both parents and children, the transition is an important part of learning how to live with a bleeding disorder. However, making decisions about childcare and finding the right caregiver can be a challenging process.

It is normal for all parents, and especially those of children with bleeding disorders, to feel anxious about starting childcare. There are many reasons why parents need day care and there are many options for child care. Interaction with new people and environments, particularly for children over 3 years of age, is important to your child's development. Good childcare helps nurture a child's sense of discovery, autonomy, happiness and well-being.

While day care centres are a popular choice for child care, other options available include a home-based carer (private or through a company like Porse), nanny or shared nanny, au pair, or friends and family. Some of these might take place at your house or at a caregivers home. Whatever you choose, be it full or part-time, it is important to find a centre/carer offering the highest quality you can for your child. It is important to find a solution that fits both your child and their personality, and also your values and budget.

Below are some steps to help in choosing childcare, in particular a day care centre, but some ideas will apply to all types of child care. These are ideas were put together by the Northern Outreach Worker and a mother of a 3-year old boy with severe haemophilia, drawing from her recent experience.

Narrow down your options

- The process of thinking about and choosing day care can be made a lot easier by attending playgroups first. This can encourage interaction from an early age, and help with trusting others when it comes time for day care.
- Research and find what different childcare options there are in your area – home care, day care centres or a nanny. Think about what would best suit you and your child and why. ***"We started by looking online to see what all the options were in our area. At first I wanted home-based care but none of them felt right for me as they seemed to do so many activities out and about, so I ended up looking into day care centres instead as I wanted my child to settle into one place and know his surroundings."***
- Your neighbourhood and community can often be the best place to start your search. Talk with other parents in the area about which day care providers they find useful; consult your Outreach Worker (as they may know of others who have used a day care in the same area), and ask doctors, co-workers and friends and family for advice and recommendations about which providers to look at first. ***"We asked around friends and families which helped us narrow our decisions down".***

Visit your preferred choices

- Once you have made a short-list of potential centres/carer, make sure you visit them in person. ***"We did a drive by first to see if their playground looked safe, then the ones we liked the look of we rang and made an appointment and went for a general visit to check them out. We didn't talk about the bleeding disorder at this time; we just introduced our child and wanted to get the vibe of the centre and the staff."*** An appointment is not always necessary for some day care centres and 'dropping-in' might give you a better sense of day to day operation that might be different from a formal tour. Check their websites or call to check their policies.

- When you visit your top choices personally interview the people who will be caring for your child. Ask questions such as:
  - What is your approach to childcare?
  - What is the quality and age-appropriateness of play equipment, both indoor and outdoor?
  - What activities do you offer?
  - Which activities are free play and which are supervised?
  - What qualifications do the staff have?
  - Do the caregivers have training in first-aid?
  - What is the caregiver to child ratio?
  - Are you willing to learn about my child's special healthcare needs?
  - Would you be willing to make safety and childproofing modifications to meet my child's needs?

It is especially important to have these conversations and see what their willingness is to understand your child's bleeding disorder.

- In your visit leave enough time to walk around and spend time watching the staff interact with the children and also check the physical environment. This can help you decide if the centre will suit your child's requirements, and whether the schedule is responsive to your child's needs. Ask yourself if your child will thrive in this setting? You can learn a lot about a centre just by watching. ***"It was probably the most important thing for us to see how the staff connected to our child and how they interacted with him and cared for him."***
- Identify the overall strengths and shortcomings of each centre and their caregivers. The ideal choice will vary according to your child's age, skills, interests and personality traits. It is important that you have a strong feeling of trust and confidence in the individuals and the centre.

Educate child carers about bleeding disorders

- Once you have chosen a child carer and agreed on a schedule and start day, start talking with the provider to dispel the myths associated with your child's bleeding disorder. Educate them, give them some resources and ensure they know about:
  - How your child's bleeding disorder is treated
  - That a small scrape or cut is not life-threatening and basic first aid should be the same as for other children
  - Treating nose bleeds (if applicable)
  - Recognising joint and internal bleeds

- When to contact you
- What to do in case of emergencies
- Contact details for you and the HTC. Provide them with a written list.

Your Outreach Worker can also go and talk to the centre with you.

"We got Sarah along to talk to them and give some resources. We also told them to call us any time for any little thing and let them know we live close by and would be available. That way we could slowly educate them and build their confidence. I also stayed with our child for the first week at the centre just to make sure everyone (son, staff and mum) felt comfortable."

Settling in

- It will take time and practice for you, your child and a new caregiver to grow confident with a new childcare arrangement. Tell your child what to expect. Explain that you will be apart for a period of time and describe the fun activities to be enjoyed with the caregiver. Reassure your child that you will be back.
- Introduce the child care gradually. Start with a short visit together, and then ease into short periods of time apart. Gradually build up to the child care arrangement. Allow time for your child to adjust. If allowed, let your child bring a special toy or blanket to give them a sense of security and familiarity.

"It was a very nervous time for us when our son started day care. It was the first time he was away from us. It took a good three months to feel comfortable. Although we were so worried to start with we knew that we had to do it and that it would be good for our son – he needed to make friends and have some freedom. It was the best thing that could have happened and we want everyone to know it gets easier with time. He loves going to day care now and it has given me (mum) time to start working again and spend more quality time with our other children too."

For further information contact your Haemophilia Outreach Worker (contact details are on the inside cover of Bloodline or at www.haemophilia.org.nz). You may also wish to visit:

- Childcare online - a directory of childcare centres in New Zealand. ***http://www.childcareonline.co.nz/***
- Choosing Quality Child Care – article by the Brainwave Trust. ***http://www.brainwave.org.nz/wp-content/uploads/2012/04/Choosing-Quality-Child-Care.pdf***
- Living with von Willebrand disease – Child care and schooling h***ttp://www.hemophilia.ca/en/bleeding-disorders/von-willebrand-disease/living-with-von-willebrand-disease/child-care-and-schooling/***

Letters to the Editor
Pregnancy and Birth in women with Bleeding Disorders

There was a story in the last magazine about pregnancy and bleeding disorders which seemed to focus quite a lot on Haemophilia and only skimmed the surface with vWD…. with some types of vWD our platelet activity gets even lower during pregnancy. So I had to have platelet transfusions and [my son] was also taken up to the special care ward for 3 days and needed platelet transfusions as well. And of course there were other complications.

I feel being pregnant and delivering babies is one of the worst times to have vWD, especially when it has not been diagnosed correctly.

- Jessica, Northern

Thank you for your feedback, the article should have included more vWD specific information as it does present particular problems. Women with vWD require monitoring both during and after pregnancy. Unfortunately, there is no definitive consensus on the optimal management of women with vWD during pregnancy.

There is a progressive increase in FVIII and VWF levels during normal pregnancy in most women with vWD, but this depends on both the type and severity of the bleeding disorder. Most women with type 1 vWD have a progressive increase in FVIII and VWF levels into the normal non-pregnant range, which may mask the diagnosis during pregnancy. In women with Type 2 vWD, any increase in VWF production does not help activity because the VWF does not work correctly and can cause further problems. Most women with type 3 vWD have no improvement in FVIII or VWF levels during pregnancy and require treatment for all types of delivery.

Because of the rapid fall in FVIII and VWF levels after delivery, women with all types of vWD are at substantial risk for postpartum haemorrhage, especially around days 4 and 5 after birth. Because of this risk it is recommended that a delivery plan with input from the haemophilia centre be in place and that all women with vWD should deliver their babies in an obstetric unit, which can easily and quickly access the facilities of a Haemophilia Centre if needed. The risk is higher in those with type 2 and 3 vWD and can persist for several weeks after delivery.

The management of some type 2 variants of vWD present special problems during pregnancy. Treatment will be required if an episiotomy is performed to assist delivery or for other operative delivery. Women with Type 2N are often misdiagnosed as being carriers of haemophilia A because of their very low FVIII levels (due to the increased binding activity of their VWF). This leads to a very high risk of postpartum haemorrhage and often requires prophylaxis for delivery. Type 2b often results in thrombocytopenia (low blood platelet levels) during pregnancy. The severity depends on the person and prophylaxis before delivery may be needed to avoid postpartum haemorrhage.

Other pregnancy-associated bleeding reported in women with vWD includes extensive bruising and haematomas at intramuscular injection, episiotomy and surgical wound sites. Delayed acute bleeding from episiotomy wounds, severe enough to require transfusion, may also occur.

Although epidural anaesthesia for pain relief may be considered for use in the majority of women with type 1 vWD whose levels have risen to within the normal range during the pregnancy, it should not be undertaken lightly and without due consideration to VWF levels, platelet levels and risk of spinal bleeding. Epidural anaesthesia is not generally recommended for use in women with types 2 or 3 vWD.

References:

- Kujovich JL. von Willebrand disease and pregnancy. J Thromb Haemost 2005; 3: 246–53.
- Pasi KJ, Collins PW, Keeling DM, et al. Management of von Willebrand disease: a guideline from the UK Haemophilia Centre Doctors' Organization. Haemophilia 2004; 10: 218–231.

It is paramount that people considering having children with haemophilia have the correct, accurate information. I feel that in its' context, in the second column 3rd paragraph down, pg 13 of March 2014 Bloodline - should have contained the percentage at which ICH [intracranial haemorrhage] does occur.

The previous sentence speaks of circumcision bleeding being at about 50 %, then says that ICH is far fewer. Indeed greatly far fewer, being only 3% of normal vaginal deliveries, and of that 3% the majority being caused by intervention (forcepts / ventouse) . It is our duty to provide the correct information, and had I have read that without proper information, I would fear the statistic was far greater.

- Caroline, Northern

Thank you, most recent studies do show that 3.5–4.0% of all boys with haemophilia born in countries with a good standard of health care experience a head or brain bleed (ICH) during the neonatal period (up to the age of 3 months). While this seems low, this is still considerably higher than expected in the general population. By comparison, the the highest risk of ICH in the non-haemophilia population is associated with vacuum extraction/ventouse with an incidence of 1 in 860 deliveries (0.1%). Other methods of delivery have even lower risk.

The frequency of ICH in newborns depends on many general issues, such as the health care status of the population studied; maternal issues, such as knowledge of carrier status and prenatal diagnosis; obstetric issues, such as guidelines for the management of carrier women and mode of delivery; and finally, surveillance and routine management of the newborn with known or suspected haemophilia. Despite variations between centres and countries, a review of the literature demonstrates that ICH in the newborn with haemophilia remains a problem that needs to be addressed with both education and research efforts.

Not to alarm parents, but mothers who know they carry the gene for haemophilia should understand the signs of a head bleed in an infant and be aware that any unusual behaviour should be brought to the attention of their haemophilia treatment centre. Although symptoms can vary, parents should know to watch for excess irritability and sleepiness, irregular breathing, seizures, vomiting and difficulty feeding, or lethargy.

References:

- Ljung RC. Intracranial haemorrhage in haemophilia A and B. Br J Haematol. 2008 Feb;140(4):378-84.

Market Research Opportunity: Would you like to earn $100?

- **Are you an adult with severe haemophilia A?**
- **Or are you the primary carer of a child with severe haemophilia A?**
- **Can you spare us 50-60 minutes of your time on the phone (at their expense)?**

If so, then a market research company would like to talk to you as part of an information gathering project on severe haemophilia A treatment and treatment choices.

The interviews are completely confidential and at a time of your choosing.

The interviews are conducted by an independent, experienced healthcare interviewer.

To thank you for your time, they will send you a $100 Progressive voucher which can be used Nationwide at any Countdown/Freshchoice/Supervalue store.

If you would be interested in participating, please send an email to: Maxine on ***max@maxpower.com.au*** stating your name and age and they will contact you to organise an interview time at your convenience.

If you are a person with moderate haemophilia A but a severe phenotype, they would also be interested to speak with you. So please drop them a line.

Editor's Note: *Please note that HFNZ are not promoting this research just providing information about the opportunity to participate. The research is being conducted by a third party market research agency on behalf of Baxter Healthcare. All identifying patient details will remain confidential and will not be passed on to Baxter at any point.*

MRG Reports

Southern

By James Poff

Dear Southern MRG Members (& the wider HFNZ Family).

It has been a busy last few months here in the Mighty South.

The team came together and once again a succesful fundraising night was held in conjuction with Heather Giles & Brick Road productions.

It was a great night had by all, members ran the raffles and the bar as well as undertaking the sales of tickets prior to the event. Great job with sales from Alex and family and thanks to Harrison for working the raffle! As a consequence over $1300.00 was raised.

Several members recently travelled to Melbourne for the WFH 2014 conference, I was one of those lucky enough to attend this event and i can honestly say that is was a great experience.

Lots of information and learnings.

Upcoming Southern Events

- Take a kid to Footy proposed date is sometime in July
- Southern Family camp 3rd to 7th October 2014 - Tea Pot valley Christian Camp in Nelson.

Now that the colder weather has hit - stay warm.

Upcoming Northern Events

Contact Sarah, Northern Outreach Worker, or a member of the Northern committee for more information.

- Rock climbing Event, Sunday 29th June 2014. Lunch at 12:30 followed by Rock Climbing 1:30 pm
- Join Northern for a fun family day trying out gymnastics. 12-4 pm, Saturday 19th July Includes lunch, organised activities and games and free time on the equipment.

Upcoming Central Events

Contact Lynne, Central Outreach Worker, or a member of the Central committee for more information.

- Join Central Members in Wanganui for their Winter Escape over the weekend of 5-7 September, 2014.

Upcoming Midland Events

Contact Joy, Midland Outreach Worker, or a member of the Midland committee for more information.

- Hobbiton Move Set Tour, Lunch and Midland Annual General Meeting - 29th June 10.30am, Hobbiton, 501 Buckland Rd, Matamata 3472. RSVP 22nd June to Wendy 07 3481544, 0276651782 or wannabe1@xtra.co.nz

HFNZ have a New Bank Account!

As of 1 July 2014, HFNZ will have a new bank account.

If you are making a payment or donation through online banking please contact HFNZ Administrator Leanne Pearce for the new account details *(leanne@haemophilia.org.nz , 03 371 7477 or 021 666 006)*.

Honour the memory of a loved one or recognise the unique bond you have formed with HFNZ to make a difference to the quality of life of people with bleeding disorders.

Information on making a bequest in your will to the HFNZ can be found at ***www.haemophilia.org.nz***

give a little

Give a little? Give a lot!

Charitable donations to HFNZ can now be made online at:
www.givealittle.co.nz/org/haemophilia

HFNZ News

An admin milestone

Leanne Pearce, HFNZ's Office Administrator, celebrates 10 years of working with HFNZ in 2014. Leanne is really the glue that holds many of the organisation's functions together and keeps us running day-to-day. Thank you Leanne for all your hard work and time over the last decade, and we hope for many years to come.

Introducing Mrs Sarah Elliott

Our fantastic Northern Outreach Worker Sarah married in February this year. As such she has changed her name from Sarah Preston to Elliott. All her contact details stay the same, including her email ***sarah@haemophilia.org.nz***

On behalf of everyone at HFNZ, we extend a heartfelt congratulation to Sarah and Fraser. We will wish many happy years together.

WFH 2014 WORLD CONGRESS

Melbourne, Australia • May 11-15

CONTENTS

The WFH 2014 World Congress took place in Melbourne, Australia, May 11-15, 2014.

The Congress is the largest international meeting dedicated to haemophilia, von Willebrand disease, rare factor deficiencies, and inherited platelet disorders. Over 4,000 attendees from more than 130 countries attended the conference, which took place at the state of the art Melbourne Convention and Exhibition Centre at Southbank on the south side of the Yarra River.

The WFH 2014 World Congress is the global meeting place for people with bleeding disorders and their families. Congress attendees also included haematologists, paediatricians, orthopaedic surgeons, physiatrists, physiotherapists, dentists, nurses, social workers, psychologists, geneticists, laboratory technicians, researchers, and scientists, who, together, represent the comprehensive care team for people with bleeding disorders.

The Congress included a robust medical program, the latest scientific and clinical developments in diagnostics, disease management, and research. In addition, there were multidisciplinary programs that covered emerging topics related to inherited bleeding disorders. Over the next several pages you will find reports on most of the sessions presented at Congress as reported by HFNZ delegates.

Having a WFH Congress so close by in Australia was a rare opportunity for the New Zealand bleeding disorders community. Never before have so many members of HFNZ, staff and their healthcare workers been able to all attend a Congress. It was an amazing chance for learning and networking and the Kiwis certainly made their mark – from our distinctive outfits at the opening ceremony and cultural evening to taking home the prize for the most donations towards the WFH Close the Gap country with the heart initiative.

WFH President Alain Weill issued a challenge to all participants in attendance, "Whether you are a scientist, a journalist, a person with a bleeding disorder, a government official or a business leader, what can you do, today, to increase the number of people who have access to treatment?". We challenge you as you read the Congress reports to not only think about this question but also how you can make a difference to care and the community here in New Zealand.

CONGRATULATIONS NEW ZEALAND !

The Close the Gap campaign has a goal of raising $500,000 by the end of 2014. This important program aims increase diagnose of new patients, especially in the world's most impoverished countries. At the Congress, the WFH booth featured a giant World Map which highlighted the state of diagnosis in different areas across the globe. Every Congress delegate who made a donation received a pin to place in their country on the World Map.

At the farewell dinner, New Zealand was awarded with the WFH World Cup for the country with the biggest heart for making the highest number of donations per capita. It was a proud achievement that inspired an impromptu haka – and became a memorable moment for delegates from around the globe.

HFNZ will also receive a $500 grant from the Trace Research Group for having the highest number of people sign-up for their market research programme.

THANK YOU !!

Thank you to all the members across the country who volunteered at events like Armageddon, ran raffles, and pitched in to help raise funds for the last 4 years to send delegates to the WFH World Congress in Melbourne. It truly was a rare opportunity to have the biggest international meeting of the bleeding disorders community so close to New Zealand and HFNZ is so excited that so many members and staff were able to attend.

Also a big thank you to CSL Behring, Novo Nordisk, Bayer, and Pfizer for their grants towards helping members and staff participate in this amazing educational opportunity. And of course thank you to the World Federation of Haemophilia for sponsoring Te Whainoa Te Wiata to attend the pre-Congress NMO Training and Courtney Stevens' Youth Scholarship.

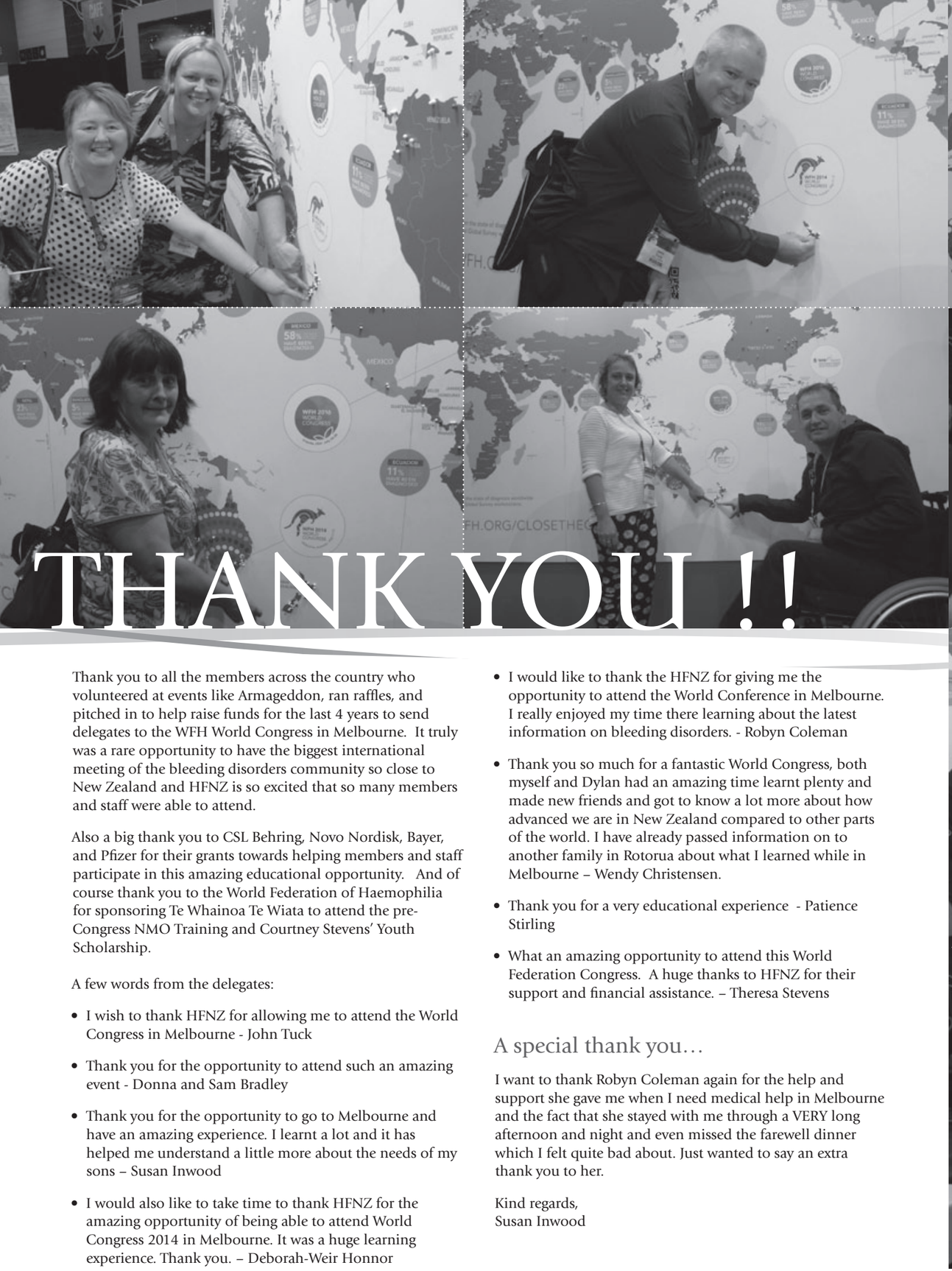
A few words from the delegates:

- I wish to thank HFNZ for allowing me to attend the World Congress in Melbourne - John Tuck
- Thank you for the opportunity to attend such an amazing event - Donna and Sam Bradley
- Thank you for the opportunity to go to Melbourne and have an amazing experience. I learnt a lot and it has helped me understand a little more about the needs of my sons – Susan Inwood
- I would also like to take time to thank HFNZ for the amazing opportunity of being able to attend World Congress 2014 in Melbourne. It was a huge learning experience. Thank you. – Deborah-Weir Honnor
- I would like to thank the HFNZ for giving me the opportunity to attend the World Conference in Melbourne. I really enjoyed my time there learning about the latest information on bleeding disorders. - Robyn Coleman
- Thank you so much for a fantastic World Congress, both myself and Dylan had an amazing time learnt plenty and made new friends and got to know a lot more about how advanced we are in New Zealand compared to other parts of the world. I have already passed information on to another family in Rotorua about what I learned while in Melbourne – Wendy Christensen.
- Thank you for a very educational experience - Patience Stirling
- What an amazing opportunity to attend this World Federation Congress. A huge thanks to HFNZ for their support and financial assistance. – Theresa Stevens

A special thank you…

I want to thank Robyn Coleman again for the help and support she gave me when I need medical help in Melbourne and the fact that she stayed with me through a VERY long afternoon and night and even missed the farewell dinner which I felt quite bad about. Just wanted to say an extra thank you to her.

Kind regards,
Susan Inwood



Medical Advances and Treatment

Non- Adherence – Explanation and Strategies to overcome it

By Kyle Cunningham

The session was chaired by Tom Shindl (HFAustria) and the speakers were Alfonso Iorio, McMaster University, Canada; Susan Cutter, Assistant Director Haemophilia and Thrombosis Centre, Pennsylvania, USA; Kate Khair, Nurse Consultant, Great Ormond St Hospital , London, UK, and; Robert Mccabe from the Haemophilia Foundation of Australia.

Non-Adherence and its impact on treatment efficacy

Alfonso Iorio began his topic with the disclaimer that many of the ideas he will cover are only relevant to patients receiving care in a first world country (England, Australia, NZ, etc). I feel this is a good way to start as it informs the audience that while the issues addressed are genuine and worthy of discussion, bigger threats loom for those patients who do not receive a high quality of healthcare.

Our speaker goes on to discuss the difficulties of quantifying adherence, "If there is a misunderstanding of the goal then adherence cannot be measured". This make a lot of sense, if a patient has a different idea about their treatment plan than their treatment centre does and there is no communication in the interim, the data will be useless. A patient can adhere perfectly to the wrong treatment plan. These are issues were addressed in slightly more depth by further speakers.

Our first speaker goes on to say that "Adherence is a process directed outcome, not a patient directed outcome". I believe that this means it takes a team working together to keep the patient adhering to their treatment plan. The physician, the haemotologist, the clinic nurses, the physiotherapist and, finally, the patient, all play their part in keeping everything going smoothly. The speakers surmised that "The quality of the relationship with Health Centre staff is directly associated with higher adherence." This seems simple enough - the more involved you are with your health centre, the more likely you are to stick with your treatment plan. The key to developing this involvement is communication; other speakers will go on to tell us. Iorio is more concerned with the quantifying of adherence and the difficulties that arise, he gives the example that many experienced nurses can still not judge an adherent patient when matched with treatment logs.

Our speaker surmises that current methods of quantifying adherence are subjective, slightly ineffective at best, and outright unhelpful at worst.

See more from Alfonso Iorio at -- Haemophilia.mcmaster.ca

Tools to assess adherence

Susan Cutter began her topic with a similar disclaimer before delving in to her years of experience working with the bleeding community. She has some interesting ideas about adherence in general and the audience seemed a bit more receptive to her "frontlines" experience of working with patients.

She discusses the fact that initially prescription of prophylaxis was directly connected to whether or not the patient would adhere to this treatment plan. From her experience, the leading factors that affect adherence are: Gender, Race, Religion, Age. Cultural influences are a contributing factor in patient adherence. Susan gave an example of how important a patient's upbringing and religious views can be when it comes to their treatment plan. She had a 30 year old with severe haemophilia raised with some extreme views who had never set foot inside a treatment centre until he saw fit to separate from his church. Susan went on to say that while some may assume this patient would be non-adherent right of the bat due to his cultural and religious background, the man was completely adherent and the key was finding the patient's learning style and adapting HER behaviour to compliment that.

This falls along the lines of social worker skills. It seems simple but, as Susan explained, this is a condition that is never going away so interacting with a patient is not a one-time thing; it's an ongoing relationship. Developing and maintaining that relationship is key to having an adherent patient. Dealing with chronic illness means that there are many opportunities to develop this relationship.

Maintenance is just as important. Adherent patients can become non and non-adherent can become adherent given the right (or wrong) circumstances. Poor social environment can be a huge factor in a patient waning from their prophylaxis. Susan gave another example of a patient who is HIV positive, and due to unforeseen and unfortunate events in his life he had become depressed and decidedly non-adherent. She found that daily calls to his apartment reminding him to have his treatment and to see how his day is from multiple people from the team that treat him helped maintain a strained relationship at a time when he needed to be keeping up with his treatment. This "multidisciplinary" approach seems to elicit the best results from patients.

The key to the multidisciplinary approach is communication.

Communication between healthcare providers establishes a consistent framework for the patient. A consistent and flexible framework is easier to adhere to. Susan gave a few tips for working with patients:

- Ask open ended questions pertaining to their adherence (often)
- Motivational questioning: What do YOU want from a treatment plan?
- Start with getting an understanding of your patient, in their words
- Where are their family? What defines them? What are their goals?

The key is having everyone on the same page - the health care providers, the physios, the social workers, the patient and those they surround themselves with. This means maintaining communication, keeping the channels open and ultimately being able to adapt case by case, event by event. Susan agreed that there is a need for a better way at quantifying adherence but in terms of her job, "logs are imperfect but it's better than nothing"

Tools and techniques to improve adherence

Kate Khair began her talk with the statement that "We label patients as non-adherent very easily." She believes that this is a bit unjust and that there are "shades of adherence." In a recent British study, 62% of patients do treat symptoms and 70% always have the time, so where is the non-adherence coming from? Kate had a habit of asking the audience these questions and it sets up nicely for her statement "Where are the carrots?" What she meant is where is the incentive for these young men (she works with teen boys in London mainly) to be adherent? Sure it will save their joints and allow them to live a relatively normal life but some of these boys have always been on prophylaxis so the damage done to their bodies is not that great and some will have experienced very few bleeds in their lifetime. Kate states that healthcare professionals really need to look at what we are prescribing the patient, from every angle – socially, psychologically and physically.

For some, the worst part about having haemophilia is having to stick a needle in their arm every second day. This is a first world problem to be sure but it is an issue none the less. So understanding the needs of a young adult is just as important as understanding the needs of the person with haemophilia.

There are many different reasons for bleeding to continue with a prophylaxis plan prescribed. I t is too simple to just put it down to non-adherence every time. Due to this, Kate felt that there is an issue of overtreating adherence issues and too much time spent worrying about this side of the issue.

Kate also mentioned the emerging technology developed for keeping track of patient prophylaxis and bleed patterns throughout the year and the pros and cons of such technology. Having your patient hard-lined to the treatment centre through an App is a great idea as it allows you to instantly pull up a patient's treatment history and when they last had a bleed so on and so forth. However, our speaker sae it as a double-edged sword in that, aside from confidentiality issues, in her experience having the App actually meant less person to person communication between patients and providers . The patients or the families of the patients no longer call when a bleed is taking place or when a patient has had an accident because they assume the treatment centre knows already as they have put the data into their App. This can cause some rather obvious issues, issues that can be resolved with a simple phone call - a phone call seemingly made obsolete by the new Apps.

Kate concluded that in order to maintain better patient support and better patient adherence an

understanding of the incentive is key. "Where is the carrot for these boys?" Just like our previous speaker, Kate believes that intercommunication between the providers and an inherent multidisciplinary approach is the best way to lay a consistent path for the patient to follow, with easy access to all of those involved when things go south. Prophylaxis use is driven by the belief in the necessity of treatment. Having a patients girlfriend constantly at his heels to have his treatment can be more powerful than any doctor. Having role models within the bleeding community is another example. Patients will adhere if they can aspire. To finish up Kate mentioned that when all is said and done, she would rather her boys just focus on having their treatment and making good decisions than stressing about a log book for data entry no one will see.

Roleplay between Kate Khair and Robert Mccabe

This was more humorous than informative but it was still interesting (and a little cringe-worthy) to see the how to and how to not behave with a non-adherent young haemophilia sufferer.

It was basically a role play of everything the speakers had mentioned, the importance of OPEN ENDED questions, understanding the patient's needs and expectations, delving slightly deeper than "Are you having your treatment-Y/N/M? "

It also showed how easy it can be to write off adolescent absent-mindedness as genuine protracted non-adherence. Sometimes young sufferers reaching their adulthood just need a good discussion about responsibility and discipline when it comes to their medication. Getting the people around the patient involved with the community can be very productive, just ask how many wives and partners know when their bleeder is having an issue or missed their treatment.

This was a good opportunity to see how these interactions take place and it made me thankful for all of the people involved in my haemophilia care who do such a terrific job, as I am yet to meet anyone who was quite as clueless as Kate's (fake) Haemo nurse.

Conclusion

This was a very interesting series of lectures; I feel the speakers did a terrific job of relaying their particular expertise into an easily digestible format, much more so than many other sessions I attended. I personally feel the best way to deal with a "non-adherent" is for a "non-adherent" to come to events like these and meet people who have not had prophylaxis all their lives and see how much damage even little bleeds into joints can do over time. Or one step further and interact with people who will NEVER get prophylaxis in their lifetime. An informative and very understandable session.

Adapting Prophylaxis Regimen to Fit the Individual Patient's Needs

Everyone is unique, so no one person with haemophilia with bleed is treated exactly the same as anyone else. So one size does not fit all!

Each individual patient needs to have their prophylaxis regime considered separately, taking into account to their personal bleeding patterns, joint status, adherence to treatments, and levels of factor.

With trials being held worldwide and more clinicians adopting this approach, patients could soon see that it can be a useful strategy to maintain their target joints and hopefully make the bleeds less of an issue. This approach can result in safer and more efficient treatment, care and health-related quality of life with overall benefits to the men involved.

By talking to the patients and getting their input, this can add a valuable insight into how, when and where. This regimen has also been proven to help the individual maintain a more active lifestyle as they can rely more on carrying on with physical activities and feeling more secure with the knowledge of the prophylaxis on board.

Prophylaxis in Real Life Scenarios

By Lynne Campbell

Craig Kessler chaired this session in which three speakers provided an insight into how prophylaxis plays an important role in the quality of life for people with severe haemophilia.

A description was provided of a comparative study focussing on intermediate versus high-dose prophylaxis for severe haemophilia and involving patients from Netherlands and Sweden. The premise was that a higher dose resulted in fewer bleeds in anticipation of a better joint health outcome.

It was noted that the effect of both aging and of adherence contributed to the overall joint health of a patient. Unfortunately the data analysis proved inconclusive because of differences between the two comparative groups. In Sweden prophylaxis continues into adulthood.

The premise is that if we start patients on prophylaxis early then at age 80 years their joint health will be such that they will be able to get down on the floor and play with their grandchildren.

In reality different patients respond differently, so patient response is individualised. Not everyone needs high dose prophylaxis. High dose prophylaxis may be effective in preserving joint health in patients greater than 24 years of age. More evidence is needed.

Studies suggest that primary prophylaxis in patients without inhibitors, started at an early age, improves long term joint outcome. Primary prophylaxis prevents recurrent bleeding and chronic arthropathy. Secondary prophylaxis slows, but may not prevent, ongoing joint damage.

Consideration was also given to use of prophylaxis in patients with inhibitors. The studies described still had many unanswered questions; however, prophylaxis is appropriate in inhibitor patients where there is frequent bleeding and a need for aggressive rehabilitation. Inhibitor bypassing therapy remains very expensive. It will always be more expensive to use prophylaxis over on demand treatment. The use of prophylaxis in patients with inhibitors remains controversial.

Another aspect discussed was the situation where target joints continue to bleed with prophylaxis. The issue was whether we should we be enhancing with adjunctive medication such as antifibrinolytics as potentially there is some thrombotic risk.

Another comparative study considered the impact of activity on increased bleeding risk. Safe sports participation involves balancing the benefits of fitness and risks of bleeding. Essentially activity increases the risk of bleeding by 43%. With factor on board this risk decreases to 6% with one prophylactic dose.

The effect of timing of prophylaxis was also investigated. By modelling a 14 year old keen snowboarder, it was found that the relative risk of snowboarding for a weekend increased bleeding risk by 41%. If he sits on the couch and is active only at the weekend his bleeding risk increases by 6%. If he stacks his prophylaxis dose time to coincide with the weekend activity the risk of a bleed decreases to 2%.

It is important to note that often very active 14 year olds incur relatively low increased risk of bleeding because they are actually active several times per week. It may be advantageous to have a lower dose of factor several times per week as prophylaxis and stack the dose immediately before a high risk activity in order to ensure adequate clotting factor levels in the blood in order to provide maximum protection from bleeding.

It is equally important to note that category 3 activities (where there is a risk of high impact collision e.g., ruby) the odds ratio for bleeding risk remains high after prophylaxis. As the efficacy of prophylaxis reduces over time, prophylaxis should be infused immediately prior to participating in physical exertion.

Strengthening multidisciplinary teams to provide inter-professional care

By Stephanie Coulman

This session gave some insight into working with the different members of your treatment team.

In Facilitating communication when working together, speaker Maureen Spilsbury used the analogy of an elephant to describe what a team is; it is many things to many people.

Using a case study of a man with haemophilia who has an elbow bleed, he and his care team will have different concerns and priorities. The patient will be worried about taking time off work to get treatment and pain levels. The medical team will be wondering if it is a bleed or is it synovitis? The nursing team will want to know about his treatment, does he have product at home? The physiotherapist will want to see him and feel the joint. The psychologist will want to know how he has been dealing with the bleed and what else is happening in his life right now. The social worker will wonder how is he getting to hospital, will he need a medical certificate. even if there is a cat or canary locked at home or is there a cat and a canary?

Maureen talked about how a good team communicates and described the communication of the nursing team she works in. For example they hold meetings, a weekly team email and a monthly centre meeting.

Kuixing (Jessica) Li, Hemophilia Clinic & Department of Hematology, Peking Union Medical College spoke about Effective multi-professionals: teamwork stories from a nurse.

Jessica is a haemophilia nurse in China and came to Melbourne in 1996 on a WFH scholarship to learn the skills to set up a Haemophilia Treatment Centre (HTC) in her hospital. She spoke with emotion about how difficult it was for her to

take new ideas back to China and try to make changes to a system. Jessica said nurses do not question doctors in China and doctors wouldn't normally listen to new ideas from a nurse.

It struck me that Jessica is stretched in her role which goes beyond medical nursing. Although she did achieve her goal and did set up an HTC it is still without a social worker as there is no government funding for this position. Jessica also has to fulfil the roles of administrator for the HTC and school/employment support for her patients. She sees her role as a bridge for patients to lobby government policy, access to product, insurance information, housing advocacy, other medical needs and pharmaceuticals.

In Effective teamwork: stories from a psychologist, Desdemona Chong spoke about how psychology can help people with haemophilia (PWH). She noted that 1 in 7 people in Australia will have a depressive episode and this is more prevalent in people with chronic conditions.

She discussed a case study of a 50-year old man with severe haemophilia A who was unemployed and depressed. He had multiple issues including social problems and needed joint replacements.

She used psychological interventions to build a rapport with him, validated his emotions, telephone counselling, regular follow-ups, small goal-setting. The outcomes of her work with this patient were: his depression lifted, joint replacement surgeries went well and overall he became more engaged.

She spoke about the challenges of working as a psychologist with PWH; perceived stigma of seeing a psychologist, maintaining patient confidentiality (sometimes information does need to be shared with the wider team); and assumptions that all information is shared.

Desdemona reminded the audience that psychologists can't read minds!

In Patient perspective on being a Team Member Barbara Forss, Patient Advisor from the Institute for Patient and Family-Centered Care spoke about being brought up in era when you didn't question authority. For her this included her parents and certainly doctors. This was partly why her diagnosis of severe Factor VII deficiency was not identified until she was 37 years of age. She said patients in the past were not encouraged to participate in their treatment.

Barbara had to learn how to communicate with the medical team. She recommended patients think about what they want to say at their appointments and to take a support person with them. Patients have rights to be listened to and treated with respect. They also have responsibilities - to participate in treatment, follow the treatment plan and provide all the information required.

Barbara said 300,000 people died in the USA each year due to medical misadventure, largely due to medication reconciliation, i.e., not knowing which medication they were on which caused problems when treated by doctors who didn't know have their treatment records.

Barbara's advice from a patient perspective: know what medication you are on and be the centre of your team.

Haemophilia Care - beyond the treatment guidelines

By Catriona Gordon

In this plenary session Dr Alok Srivastava discussed the need for the collection of good evidence of outcomes for people with haemophilia (PWH) receiving clotting factor concentrate therapies (CFCs). He noted that there is a wide disparity of the use of CFCs in the developed world, even with the agreed haemophilia treatment guidelines. Differences include the age of the patient when prophylaxis commences, the number (if any) of joint bleeds before prophylaxis is started and the initial level of CFC dose.

These differences, and the lack of good data being collected which include the variations mentioned above, mean that we cannot be certain of what the optimum treatment is to obtain the best outcomes for patients.

Dr Srivastava also discussed the situation in developing countries, where access to CFC is very limited. There is evidence that even having PWH on a small prophylaxis dose twice or three times a week can substantially reduce the time at risk of bleeding (when they are at <1% of factor) and create a corresponding reduction in the risk of joint and muscle bleeds. More evidence is needed to be able to provide a convincing case to the health care providers of these countries of the real long-term benefits to PWH by being treated this way.

At a most basic level, patients should have an annual joint score undertaken at their annual review, and a calculation of their ABR (annual bleeding rate). This data is important for the individual patient, as it is a useful summary of (some of) the effects of haemophilia on them in the preceding year. Of course, without accurate or nearly accurate treatment records which include records of your bleeds, this information is impossible to obtain with any degree of confidence. Treatment records really do matter! On a macro scale, this information can also be fed into a collection of the data of many PWH around the world, and over time this data will provide the evidence of the effectiveness of our care in New Zealand compared with the rest of the world.

Vein Health: Promoting Successful Treatment for Patients and Families

By Linda Dockrill

Maintaining vein health is a very important focus for someone with a bleeding disorder and in this session the challenges of accessing veins and strategies to achieving success were highlighted.

Peripheral access to veins is considered the "gold standard" as it is the easiest, fastest (once you know how) and the safest from a clinical perspective. The strategies to achieve success were listed as – using an upper limb, hydration, choosing the right vein, warm limb and stabilising the vein. A few tips from Emergency Medicine added to these to make a comprehensive list and included locating veins by "feel" (practicing this is important), using solid traction so that even those veins that roll can be held still, using a double tourniquet, and it was suggested to use surface veins on the upper arm or back of the arm (particularly for older people).

STRATEGIES FOR SUCCESSFULLY ACCESSING VEINS:

- Use an upper limb
- Make sure you are hydrated (drink water beforehand)
- Choose the right vein
- Warm your limb
- Stabilise the vein
- Locate the vein by 'feel'
- Use solid traction to hold vein still
- Use a double tourniquet
- Use surface veins on the upper arm or back of the arm, particularly for older people

A second speaker discussed Central Venous Access Devices (CVADs) and Fistula's (AVFs) which are needed when someone has poor veins, the patient has physical limitations or requires frequent infusions as with prophylaxis or immune tolerance therapy. There are many pros for having a CVAD inserted – they alleviate anxiety and stress in parents and child, allow more frequent infusions, and are easier to care for than external devices. However, the list of cons is quite extensive and includes a 30% risk of infection, an increased risk of inhibitors (if aged between 2-6 years at the time of placement) and the risk of endocarditis (a life threatening infection). Similarly, AVFs have benefits and risks and it was identified that they have a low infection rate and can be used for a long time without limiting physical activity. The main concerns seemed to be that they can cause permanent alteration of the vein or alter the growth pattern of the arm they are placed in. My summary of this is that CVADs and AVFs have a place and a purpose but venous access is the gold standard if possible. Both speakers agreed that home treatment has brought an improved quality of life with it.

The final speaker, nurse Sherry Hubble from the USA, discussed needle phobia. A phobia is a persistent, abnormal or irrational fear that compels one to avoid the situation. It can cause very real physical symptoms such as hypertension and heart palpitations. There are a variety of treatment strategies available including anaesthetic, behavioural therapies, de-sensitisation and distraction but the key message was that we can't tell who has a needle phobia by looking at them. Providing care, respect and reassurance is key.

The list of DO NOTs were:

- Do not forcibly hold down a person while sticking them;
- Do not resort to deception, threats or trickery, and;
- Do not be judgemental or make light of their fear.

These 'do not's 'need to be balanced out with providing treatment, particularly in the case of young children, as many parents commented. This nurse also talked about home treatment and the importance of teaching parents and children to treat wherever they are so that treatment is a no fuss part of everyday life for families and individuals. Sherry stated that she recommends that boys develop a second vein to use in case they can't access their main one and told us how they instituted "pick a new vein day" at camps in the US, which seemed like something we could institute in New Zealand.

Approaches to Inhibitor Management

By Lynley Scott

This session was chaired by James Munn from USA, and consisted of three sub-sessions.

Nursing: Challenges and top tips in managing tolerisation in children

Chris Guelcher, USA, presented a case study and tied in some key questions/challenges while discussing the case study.

Why do certain patients develop inhibitors? No definite answer but age of first factor exposure, treatment factors surrounding first factor exposure (i.e. trauma, how much, associated surgery, etc), race (increase inhibitor development in Hispanic and black Americans), gene mutation (increase inhibitor development in large genetic deletions), and immunological aspects.

What is the best way to eradicate inhibitors? Studies have shown eradication of inhibitors via Immune Tolerisation Therapy (ITT) is more successful in patients with peak historical titre of under 200 BU. ITT is started when the titre is under 10 BU, time between diagnosis of inhibitor to induction of ITT is less than 2 years, and the duration of inhibitor is less than 5 years.

Why doesn't ITT work for everyone? Chris didn't offer any answers to this one but did outline a prospective study which compared low dose (50u/kg 3x/week) and high dose (200u/kg/day) with a 70% success in both groups. Although the ITT took longer in the low dose group and there was increase bleeding.

What do we do when ITT is not working? Switch products (try plasma-derived) or combine ITT with immune modulation (Rituximab)

Summary of top tips:

- Delay exposure to factor when and where possible
- Avoid exposure to factor with danger signals (immune reaction)
- Identify inhibitors early
- Eradication is ultimate goal
- Participate in studies

Top tips for people with haemophilia and inhibitors when they bleed:

- Minimise risk of bleeds
- Prophylaxis with NovoSeven or FEIBA
- Identify potential bleeds early
- P.R.I.C.E.
- Involve multidisciplinary team

Chris also suggested using peripheral venous access when possible for ITT, but the best option should be determined on a case by case basis. He also suggested instilling a healthy respect for CVAD and to treat them with utmost care to prevent infections and complications.

Physiotherapy: Breaking the cycle of bleeding and rehabilitating bleeding episodes for children with inhibitors

Ian d'Young, from New Zealand presented a case study and outlined some of the challenges in treating bleeds in people with inhibitors:

- Early physiotherapy and rehabilitation is needed with all bleeds.
- Managing perceptions and expectations, i.e., rehab is ongoing despite surgical procedures to repair damage done, being active does not necessarily mean that targeted physiotherapy is not important.
- Rehabilitation is long term, not just when things have returned to 'normal'.

Psychosocial issues and coping strategies for patients and families dealing with an inhibitor

Susan Cutter, USA, began her presentation by discussing quality of life (QoL) data comparing PWH with inhibitors (PwHwI) to those without inhibitors. PwHwI exhibited:

- Decrease QoL in physical domains but similar in mental health domain.
- Increase in hospital admissions
- Increased pain
- Increase joint disease/Ortho issues
- Decreased mobility and increased impairment
- No difference in employment.

Lack of access to factor results in a negative impact on QoL. Interestingly all studies have been done in 'developed' countries, but what is the impact of inhibitors in 'undeveloped' countries?

The economic burden associated with inhibitors not only causes increased hospital/medical bills but also affects the family immediately as there is an increased number of caregivers not working due to the child with inhibitors' health needs.

The impact on caregivers of PwHwI is huge:

- Caregivers report they just feel like they have mastered haemophilia but then feel like a double whammy has struck with inhibitor diagnosis.
- Caregivers report they no longer feel they fit into the haemophilia community.
- Parents' perceptions influence their children's QoL.
- Inhibitors affect their competency in mastering the complexity of their child's care (i.e., managing bleeds, ITT, venous access)
- Increase concern for child's future
- Increased impact on socialisation
- Increased mortality and morbidity issues
- Increased 'burdens of care' in domains of finance, social, general negative and siblings.
- Increased anxiety/stress/disruption on bleed days.

Susan discussed a study that interviewed adults with life-long inhibitors. Some comments made included "felt like a double whammy", "has made me stronger", and 'has been a blessing." They discussed their biggest concerns included: chronic pain, mortality, limitations on all aspects, venous access concerns, insurance coverage, and increased disabilities as they age. All but one participant said the impact inhibitors had had on their self-esteem including feeling less 'whole', the inability to play sports, inability to continue working, difficulty providing for family, feeling isolated from others, difficulty with mobility ("feeling completely crippled"), and lacking a sense of normalcy as a child. However, comments also made were: "Overcoming these challenges on a daily basis can do nothing but give a person confidence and a unique perspective on life..." and "I wanted to make sure I was living". The study found that those who are not completely defined by their haemophilia, exhibit stronger self-esteem and more confidence.

Susan concluded by outlining strategies to assist PwHwI and those supporting them:

- Utilise QoL instruments to assess.
- Tailor interventions to results
- Normalise reactions
- Utilize peer mentoring and group educational opportunities for fostering support, decreasing isolation and enhancing info.
- Strengthen resilience by fostering flexibility, hardiness, optimism and advocacy, and health efficacy.

The Future of Haemophilia Prophylaxis with Novel Therapies

By Raukura Riwaka

Dr Mike Makris, a paediatric haematologist from Canada, spoke on looking forward to longer acting concentrates.

Past – 50 years ago haemophilia was known as a terrible disease and then on-demand treatment was gradually made available. However, progress in treatment was necessary due to severe joint damages and long hospital stays.

Present – Effective prophylaxis is able to protect against bleeds. Therefore there is less need for hospitalisation and a higher standard of care for PWH. The cost of treatment means it is unavailable in some countries creating a situation of 'the rich vs. the not so rich' in terms of quality of care and life.

Dr Makris then asked the audience if anyone never misses a treatment. Only one person raised their hand. He stated nobody is perfect and every PWH misses treatments, either because of time, inconvenience, difficulties with veins, cost or availability.

How adherent are patients to prophylaxis? The doctor affirmed it is not great. Each time a dose is missed; there are more chances of a bleed.

Future – New technologies are being developed, especially longer acting FVIII and longer acting FIX.

How will these long-acting products impact on prophylaxis? They will mean fewer infusions, even once every 10 days. The benefits of fewer infusions include less central venous lines, more convenient times for treatment, and less trips to hospital. Because the risk of bleeding is reduced and there should be less joint damage there could be opportunity to participate in more sports, however, discretion is required and contact sports should still not be recommended.

There will be more individualisation of therapy, it's not one size fits all.

The significant question of the discussion was "Who will decide who switches to longer acting product, the boy or the mother or the doctor"?

A very encouraging presentation from this specialised doctor, however, in conclusion I feel that we all know the doctors will make the final decision. Then again I feel there must be a level

of relationship that has developed between doctors, haemophilia nurses, outreach workers and the whanau to be able to make that decision on who will receive the longer acting product. So although this is a major advancement for haemophilia, there must still be the ongoing scrutiny.

Advances in Replacement Therapy - Latest Updates From Clinical Studies

By Chantal Lauzon

Several companies presented the latest data on the products they have in development during this session.

Biogen Idec

Biogen are developing a long-acting recombinant Factor VIII product with a fusion-protein to extend its half-life (rFVIII-Fc)

The Key-A-Long study had three treatment arms:

- Arm 1 - Prophylaxis every 3-5 days
- Arm 2 - Prophylaxis every 7 days
- On demand

After a 12-month pre-study period to establish baselines (bleeding rates, factor consumption, etc) for each patient they were placed on one of the treatment arms according to their bleeding tendencies. The actual interval between treatments was individualised to each patient.

Overall, a decrease in the annual bleed rate was seen across all dosing intervals.

One of the main advantages to prophylaxis with a long-acting factor concentrate is fewer infusions over time. Even in Arm 1 there was a reduction in dosing frequency compared to pre-study. The total number of infusions was reduced by at least 33%. If people moved to the 5-day interval they reduced the number of infusions by 58%. Overall FVIII consumption remained practically the same pre versus on study.

In Arm 2, the annual bleed rate in people who changed from on demand to once-weekly prophylaxis reduced from 29 to 4. Although the 7-day interval proved not ideal for regular prophylaxis with this product, it did make a big impact on the people who only previously treated on-demand and was still thought to be convenient.

rFVIII-Fc maintains higher troughs levels of FVIII than current rFVIII products. It would also seem that people with higher VWF levels at baseline could usually go to the 5-day prophylaxis schedule as this seemed to correlate with their individual half-life for the product.

Overall, compared to the pre-study period there was a reduction in bleeding rates, a reduction in infusion frequency and the same amount of factor consumed. It shows that long acting FVIII is a viable treatment option.

Bayer

Bayer presented data on BAY-94-2027, a long-acting rFVIII which retains full coagulation activity, has a longer half-life and has showed reduced immunogenicity (chance of provoking an immune response).

Phase 1 and 2/3 studies have looked at dosing up to 7-day intervals. Phase 1 studies have confirmed the safety and a half-life averaging 19 hours. Each individual patient had in improvement in half-life compared to current rFVIII products.

Individual bleeding tendency is highly variable. Even at three times per week prophylaxis with current products, 72% of patients have more than 7 bleeds per year.

In the PROTECT study, prophylaxis was tailored to the person individual bleeding tendency in the 1-week pre-study period. If a person had more than 2 bleeds per week they received a twice weekly prophylactic dosing schedule on study with a higher dose. Other participants were randomised to either a 5-day or 7-day dosing schedule.

Results showed that there were more bleeds in people on the 7-day schedule compared to the 5-day. All patients on the 5-day schedule stayed on that treatment schedule, unlike 25% of those on the 7-day arm who changed because they felt treatment was inadequate. In the 5-day arm, 44% of patients had no bleeds during the study period. In the 7-day arm, 36% of patients had no bleeds.

The overall results showed the efficacy of different regimes (2x week, 5-day, 7-day) with treatment tailored to the patient's own bleeding tendency.

A note of caution about the results, however, as other experts felt that the studies, especially the PROTECT study, were too short in length (3-6 months) to extrapolate meaningful data such as reductions in annual bleeding rate and needed longer follow-up times.

CSL Behring

CSL Behring are developing two new recombinant products to add to the plasma-derived products they already manufacture. There is a single-chain recombinant Factor VIII and a long acting recombinant Factor IX product.

For the FVIII, CSL have fused the rFVIII into a single chain. It has comparable biochemical properties to the full length rFVIII in terms of half-life and has not had any additional chemical modifications. Early studies have shown that the time spent to drop to 1% through levels were longer for the rFVIII single chain compared to ADVATE.

Their long-acting rFIX-FP (fusion protein) product is currently in Phase 3 trials. They are looking at 7-day, 10-day and 14-day prophylactic dosing schedules. It is a small study though with only 24 patients. Their rFIX-FP has shown has an improved half-life of up to five times longer than over available marketed FIX products, both plasma-derived and recombinant. They are looking at an ideal treatment interval of 14 days and will also look at up to 21 days.

With these new long-acting products, there is a call for individualised dosing intervals to suit a patient's personal half-life for the product they use.

Novo Nordisk

N8-GP is a long-acting recombinant FVIII that uses glycoPEGylation of Novo8 to extend the half-life. Although these are early results, it has been safe and well tolerated across clinical trials.

Pathfinder 2 is a pivotal Phase 3 trial. It is multi-national, open label, non-randomised and only just finished so the data is not complete. The study period is for 1 year and then longer for those in the extension trial. There is also a separate paediatric trial.

In total 270 people received doses of N8-GP on Pathfinder 2 but data was only available for 150 patients aged >12 years. There were two study arms - on demand (Arm 1, n=11) and prophylaxis every 4 days (Arm 2, n=175).

According to the data available, N8-GP offers highly effective prophylaxis with less frequent dosing. The annual bleeding rate of those in Arm 2 was 1.3 and Arm 1 was 30.9. Almost all (95%) of bleeds were treated with 1-2 injections. Mean trough level was 8%. 161 patients have had over 50 exposure days and they saw no safety concerns, just one mild transient inhibitor.

A member of the audience noted that the pegylation process interferes with some aPTT reagents in the one-stage clotting assays so it can be difficult to do post-infusion monitoring but the presenter replied that chromogenic assays not effected.

Novel Products for Haemostasis

By Chantal Lauzon

This session covered new bypassing agents, the issue of biosimilars and products that are available to treat rare bleeding disorders.

Bypassing agents

Bypassing agents are used to treat bleeding in people with haemophilia and inhibitors. There are new bypassing agents in development, ACE190 for example, that could also be used from early childhood and might also be a treatment option instead of doing Immune Tolerisation Therapy. Much more clinical development is needed however.

Biosimilars for FVIII

A similar biological or 'biosimilar' medicine is a biological medicine that is similar to another biological medicine that has already been authorised for use. Biological medicines are medicines that are made by or derived from a biological source, such as a bacterium or yeast. Comparisons can be drawn to generics for chemically-manufactured medicines.

The rationing of healthcare costs and treatment needs of low income countries are driving a desire for biosimilars that could be available at a lower cost to the original treatments.

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Photo courtesy
of John Tuck

There are other treatment areas where biosimilars are being developed but questions remain if it is an option for FVIII.

FVIII is a very complex and large molecule. Current rFVIII products should each be considered unique molecules and treatment products. Biosimilars are biologic products engineered to be highly similar to the original biologic product but they are never identical. This can be down to the complexity of the molecule or active substance or the manufacturing process. Ideally they should work the same and be just as safe though.

There are differences in producing chemical medicines versus protein based entities (i.e. non-biological compounds such as aspirin, antibiotics, vs. biological, which are derived from living organisms or recombinant DNA). Non-biologics can be duplicated fairly easily once the recipe is known. Biologicals are much harder to replicate.

It is real challenge for other companies to figure out the manufacturing process of factor products as this is usually commercially sensitive information. Some existing manufacturers are developing their own biosimilar version of their own rFVIII molecule.

The big driver for their development is a potential cost-savings, so will biosimilars reduce the cost of FVIII treatment?

The presenter thought maybe, but their acceptance by physicians might be a problem. One of the biggest concerns with biosimilars is safety and the potential for the development of inhibitors, especially as there is already a risk of inhibitor development with FVIII.

That aside, models have shown that the cost erosion of introducing 'biosimilars' is not as strong as for generic medications and only around 10-15%. This type of savings can already be obtained through using a tender process to purchase factor because that already introduces competition to the local market.

The lower costs are generally not because of savings in the manufacturing process but the streamlined regulatory process, which is less complex and requires fewer clinical trials then traditional products. This also raises concerns for many.

Because safety is a critical concern for biosimilars the extent to which biosimilars can provide safe, less expensive, as effective options for haemophilia may not be enough to justify their development. There is also a question about whether clinicians would feel ethically comfortable enrolling people on biosimilar trials.

Factor concentrates for RBD

This part of the presentation is reported with the von Willebrand Disorder and Rare Bleeding Disorder reports.

Genomics of Bleeding Disorders

By Chantal Lauzon

Genomics is a branch of molecular biology concerned with the structure, function, evolution, and mapping of genes.

Over 15 years ago people began doing mutation analysis on the genes associated with haemophilia and vWD. Although the methods are robust they can be time consuming and expensive. New technologies, such as next generation DNA sequencers, are able to analyse many regions of a gene at once. The newer approaches allow for lower cost, higher throughput mutation analysis for haemophilia A and B. They are good for analysing the samples for many patients at once; however, this means they are still better suited to research then hospital use.

In the UK, they have developed a panel which allows comparison to the genes responsible for a number of bleeding disorders at once. The developers hope that using the panel will help streamline genetic analysis as they can look at the genes for a number of disorders at the same time without having to try to amplify each gene separately.

Research using a similar process has been able to analyse DNA samples from large groups of people to identify common mutations linked to mild haemophilia and platelet disorders, which as usually harder to narrow down.

The technology in this area is developing rapidly and although best suited to research purposes at the moment, there is hope that it can be applied to patient diagnosis in the future.

It is important to have the individual mutations in your Factor VIII and IX genes analysed, where available. For example, understanding what mutations you or your child has could help estimate your/their personal risk of developing an inhibitor. People with certain genetic mutations in their haemophilia genes are more susceptible to the development of high-titre inhibitors (i.e., large rearrangements in the Factor VIII gene or certain missense mutations). In families where there is more than one person with an inhibitor, the chances of related family members with haemophilia developing an inhibitor are quite high.

The genetics associated with your immune system also plays a part in inhibitor development. Researchers cannot yet say for sure which immune system- related genes are responsible for contributing to the development of inhibitors but they have some candidates they are studying.

Living Well

Recipes for healthy living

By Te Whainoa Te Wiata

This seminar was based on in depth information about the basics of healthy living while living with a bleeding disorder. Presented by three amazing speakers Joanne Deutsche (USA) who spoke on "How nutrition affects coagulation", Kristy Wittmeier (Canada) who spoke about "Obesity and Haemophilia" and lastly an influential speech by Andrew Selvaggi (AUS) who presented "a patient's perspective" and the challenges he had to overcome while enduring a life long battle with an inhibitor. All three of these presenters were very relaxed and open in their approach towards this matter which made it very easy to process, especially for those of us who aren't really up to play with the current terminology as far as haemophilia is concerned. In the minds of these speakers the topic of healthy living within the bleeding community is a priority.

How nutrition affects coagulation

Joanne Deutsche, Family Nurse Practitioner, Oregon Health and Science University, USA.

If you have ever wondered what iron, vitamins and minerals are and the role that they play in your body, this opening part of the session would have answered a few queries. The speaker simply expanded on the basic needs the body requires in order to function smoothly. Over two billion people in the world are iron deficient and it is considered to be the most widespread nutritional disorder in the world (Joanne, Deutsche. personal communication.12/05/14). So the stresses of the daily intake of iron are important to everyday living whether you are a person suffering from a bleeding disorder or not.

The best source of iron one can sort out today is iron still associated with meats and fish (haem-iron) as they are absorbed better by the body as opposed to the iron found in vegetables (non-haem iron). Joanne also differentiated that ferritin is a protein that stores iron in the liver and

the form of iron in foods and dietary supplements, while haemoglobin is the iron-containing protein that carries oxygen to the cells.

Vitamin C (ascorbic acid) Much of the population around the world is considered to be vitamin C deficient. Hence, good intake of vitamin C along with good sources of iron are important as they are vital for healthy bones, teeth and cartilage, and it is also needed to make collagen which is found in blood vessel walls. Collagen is considered to be a coagulant activator when collagen comes in to contact with platelets. So vitamin C intake is highly recommended for people with bleeding disorders.

Vitamin D (the sunshine vitamin) Again, like before, many people across the world are vitamin D deficient. There are many variables to Vitamin D, it promotes calcium and is needed for bone growth and bone remodelling. Haemophilia is associated with low bone mineral density, so Vitamin D taken along with calcium is going to help strengthen bones.

In addition to everything are deficiencies in Calcium and Vitamin K on a global level which too can play a role in coagulation, yet in this seminar the speaker had a lot more emphasis about the intake of artificial supplements. Although stated briefly it is very basic, it is said that supplements are going to inhibit the absorption of any vitamins and minerals that one puts into their body, therefore restricting or limiting the intake of such products should be considered seriously. The main message that came out was of importance of knowing what roles vitamins, minerals and the right foods play when absorbed by the body and how they can help or inhibit everyday life.

Obesity and Haemophilia: Where are we at?

Kristy Wittmeier, University of Manitoba, Winnipeg, Manitoba, Canada.

Haemophilia, poor joint health and obesity seem to go hand in hand with each other in some sense, yet the latest research shows that there is no clear evidence that bleeding episodes are associated with obesity. What is clear is the need for serious consideration towards these three factors within the bleeding community. One approach is a personalised one, which is currently being used in Canada at this time; such an approach would better indicate the best way to deal with the issues of individuals.

In the general public most (not all) hip and knee replacements are associated with obesity, so it is believed that weight loss can improve the effects of joint health. Many people who suffer from haemophilia and various other bleeding disorders are sometimes inhibited or limited in activity, as a bleeding disorder can affect a person's activity choices and activity level which in turn can lead to poor joint health and also put them at risk of obesity. All of these factors interact with one another, so it is

difficult to pinpoint the initial problem — which highlights a need for a personalised approach. So overall health is what is needed to improve these target areas of personal well-being, and eating guides are being used to encourage a lifestyle change.

"Be critical of food industry advertising, healthy eating is a lifestyle choice which includes learning how to cook from scratch and take the time to sit down and talk with others."

This is the eating guide currently being used in Brazil, and is one of the simplest versions being used today, as most eating guides are very hard to understand. Time was taken to present some of the initiatives that are being used in Canada. Health issues such as obesity are often not the easiest thing to talk about; self-confidence issues within an individual are often the biggest barrier. The use of conversation cards to encourage discussion between patients and clinicians are being used, these cards are also to help build relationships and on-going communication between patient and clinician. Also presented were the 5 A's…

- Ask, for permission to discuss weight and readiness for change,
- Assess, health risk and route causes,
- Advise, on risk and benefits and treatment options,
- Agree, with client on realistic expectations and a plan to achieve goals,
- Assist, in addressing barriers, communication.

Finally in closing the speaker left a few take home points. There is a need to be concerned about such issues, but if the right steps are taken these personal problems are treatable in a respectful and achievable way. Long term solutions are the key, there is no overnight pill or procedure to apply to this matter, and always keeping in mind that small change can make a difference. Success is different for everyone and an individualised approach is going to be a step in the right direction to achieve what could seem impossible for an individual.

A patients perspective: How eating well and physical activity can help prevent bleeding, and improve quality of life.

Andrew Selvaggi, Australia.

Andrew Selvaggi is 26 years old and is a combination of everything that was presented and more. He is a living testament of healthy eating and physical activity all the while living with severe haemophilia A and inhibitors. Through determination, hard work and a good diet he has managed his way out of his wheelchair, lost 37 kgs and at the same time has become a great role model. Andrew is now a personal trainer and an IT specialist. He is working closely with the Haemophilia Foundation of Australia and is a prominent figure in the bleeding community of Australia. His persistence with his diet and physical activity dropped his internal bleeding rate from 1-2 bleeds a week to one bleed every 1 or 2 months. He is a living product of proper nutrition, a good exercise regime and endurance, even after a highly painful knee replacement and ankle fusion. While recovering he was still able to persist with his health regime and believes that his diet was the reason for his quick recovery. Andrew gave a great presentation and closed with his recipe for success "1 cup of exercise, 2 cups of healthy eating, 1 tablespoon of motivation, 1 teaspoon of determination and a pinch of craziness".

Ageing Gracefully With Haemophilia

By Paul Long

I wish this was one of the sessions at Congress that we found out a life changing breakthrough, but instead it was mostly more of what we already know or should know. That's not to say that the session was boring or dull, it was in my opinion a good session and I'll explain that if I can.

How do we Age Gracefully with Haemophilia? Well the question is basically the same as Ageing Gracefully.

Eat well, Sleep well, control your weight, play hard and be happy. Come on guys you know all this by now right?

So what was different for us? Well we need to plan a little better, think of the problems you might face in the future and plan for them now.

If you have mobility problems or your mobility is getting worst, then check your home for mats and the like that you may trip over or you might need to get some alterations done to your home, that sort of thing.

Health is a big thing for everyone and more so for us, more weight is harder on the joints but again, you know all about this. In our ageing years things will go better for us if we address this now, and this goes for those of you that need to put on a little weight as well.

Fitness! Fitness is important for everyone as not only will it help your health but it will also make it a lot easier to enjoy your life, enable you to get out and be active in your community, and stay in touch with people in your life.

Plan…Plan for the future, get the information on what services you may need in the future now so that you are ready for it when its time.

I'm sure you can see where this is all going by now, so I'll end this with the basic points of the session.

Plan ahead, be healthy, live life and be happy.

LOOK AFTER YOURSELVES AND EACH OTHER.

Differentiating Arthropathic Pain

By Chantal Lauzon

What is Pain? It is a warning that something is wrong. The brain interprets pain signals as danger and remembers inputs (what caused the pain last time). But the brain can be wrong about what is causing the pain. (For a good example of this check You Tube for Lorimer Moseley's snake bite story at TEDx Adelaide).

In people with bleeding disorders, pain can be caused by arthropathy (haemophilia-related arthritis) instead of bleeding and synovitis. It is very important to differentiate between them – not only because it saves money on factor products, but gives you peace of mind that you are getting the right treatment and means you can do the right type of physiotherapy.

How to differentiate between bleeding and arthropathic pain?

- Consider history of bleeding and how past bleeds in that joint have felt
- Physical examination with an ultrasound

PWH suffer arthritis at a much younger age than the general population so it sometimes not considered right away by treaters as a possible cause of pain. Awareness needs to be raised even with young PWH that they can get arthritis too.

Neither physicians nor patients always diagnose a bleed correctly. It can be difficult to distinguish the cause of the pain, especially if there is a lot of damage in the joint. In both cases you will often have swelling and loss of motion and not every joint that is hot or swollen is having a bleed; people with haemophilia can also get tendinitis, infections, broken bones, etc.

Can you tell the source of pain by the words used to describe it? Pain and how it is described seems to be highly variable to an individual. There are lots of pain questionnaires available, i.e., the McGill Pain Scale, but they don't seem to be accurate enough to distinguish between haemophilic bleed related pain and arthropathic pain. Studies have found that the words use to describe acute and chronic pain often overlap (throbbing, aching, tender…etc).

If the pain is caused by a bleed treat it as normal – factor, P.R.I.C.E., painkillers, etc. Arthritic pain needs painkillers, functional therapy and adaptive therapies. Symptom control methods for arthritic pain are often contradictory to methods for treating bleeds (i.e., heat vs. cold, use of anti-inflammatories such as COX-2 inhibitors).

The goal for treatment providers is to ensure they give the right patient the right treatment at the right time and with the right dose.

In haemophilia, the message has been factor first, investigate later. So have we created a problem? Pain signals = bleed = must treat with factor. This message is still important but especially as people age they and their carers need to consider there may be other issues to take into account.

So all of a sudden you might feel that clinicians are suggesting wait and check to see if the pain is actually arthritis and not a bleed – but what if it feels the same as bleed?! This doesn't mean they do not believe you are in pain. Over time, people with bleeding disorders have become extremely sensitive to pain and learned that factor relieves the pain and therefore it makes sense that more pain needs more factor. This is a process can sensitization. An ultrasound can be used to confirm bleeding if there is any doubt.

BJ Ramsay, haemophilia nurse at Wellington Hospital spoke about the role of the nurse in helping differentiate arthropathic from bleeding-related pain. He asked do PWH consider arthritis as a cause of pain? If the same thing has been happening to you for over 40 years and it was always caused by the same thing in the past would you consider another cause?

He mentioned how living with haemophilia is a lifelong process and the experiences and choices made in youth are carried through life in the joints of a PWH. For example, a childhood biking accident that caused a serious knee bleed can create a target joint for many years to follow.

The haemophilia nurse tends to be healthcare professional with the most contact with PWH. It is an existing trusted relationship, and one that delivers more than just physical care (they know you, your joints, your life).

Sometimes nurses need to have difficult conversations with their patients. The PWH has had haemophilia all their lives and are experts. Just because a nurse/doctor suggests that the pain is not being caused by a bleed doesn't mean they do not acknowledge there is pain. It is important to keep accurate record/reports of bleeds and treatment to help look for patterns. If you are on home treatment this does not mean you do not have to report a bleed, have it checked and then have physiotherapy to rehabilitate.

Changing long standing practices take time, but it is important for nurses to get people thinking about different sources of pain and ensure they have a good assessment to confirm/rule out bleeding.

To treat pain you need to use the best tool for the job. Factor can be a very expensive placebo if the pain is not being caused by a bleed.

Ahmad Hazri from Malaysia gave a patient perspective on differentiating pain. Ahmad is 27 years old and has severe haemophilia.

He has severe arthritis in his left elbow and left ankle. Since starting low-dose daily prophylaxis in 2002 he now has few bleeds.

To him, bleeding pain feels like repeated beating on the joint with a blunt instrument. Arthritic pain feels like repeated stabbing in the joint, but it fades with time.

For him, arthritic pain is not just from getting up in the morning or starting to walk after a period of rest but can be sudden and sharp too, like after a certain movement. An example of how he can tell the difference is if he can hobble if it is arthritic pain in the ankle as he needs to warm it up but if he is having a bleed in his ankle he cannot weight bear on that leg.

He keeps moving as he finds it helps with joint mobility. He is a swimming instructor and he finds swimming really helps with the pain from arthritis. If he is bleeding he can't swim as, for example, he can't extend arms.

During the question and answer period a few more tips were shared:

- Timescale is a good way to differentiate pain: if it is a bleed the pain gets worse and worse; if it is arthritis is can be describes more as a steady 'ticking' or gets better after moving for a while.
- If a doctor, nurse or physiotherapist starts asking questions about your pain they may be trying to differentiate the cause of the pain - it doesn't mean they do not believe you are in pain!
- Everyone can do some form of exercise. Exercise can definitely be helpful to help recover from a bleed, with arthritic pain and help prevent future bleeds. Keeping active can also help the brain cope with pain.

Chronic pain management

By Sam and Donna Bradley

We were privileged to attend the Chronic Pain Management seminar chaired by New Zealand's own Ian D'Young. The four four speakers had common themes:

- Everyone responds to pain differently because pain is subjective.
- Drug effectiveness for chronic pain is questionable.
- Pain reduces function.
- Pain affects our quality of life.
- Pain and fear avoidance of exercise is unhelpful.
- Pain does require a team to manage it.
- Compliance of prescribed treatments varies.
- There is not enough information about chronic pain.

Dr David Butler, Australian Pain Clinician and Educator, explained that pain is the most common and costly health problem on the planet. He stated that the brain cannot feel pain but has learnt to construct pain from a neurosignature when damage occurs. This is effective for acute pain that requires a find it- fix it model, however, this doesn't work in chronic pain.

There are over 400 parts of the brain that involves pain and all senses can contribute. It only requires one sense to ignite a pain response and nerves can only signal damage. Pain is how the brain learns to determine nerve and sense signals. Dr Butler spoke about the phantoms limb test. It is well known that pain can still be experienced by the brain in a limb that has been removed, giving weight to his learned response theory. He stated that there are three inputs to pain: the Biological (What is happening physically), Psychological (How our brain interprets pain) and Social (What is happening in our lives).

Helpful therapies for pain include: Restoration of normal movement, reducing unnecessary fear to facilitate movement, limit unhelpful metaphors, provide empowering education, liking and re-embodying the body part, knowledge, perception, social interactions, belief systems, humour, intimacy and diet.

Kate McCallum, New Zealand Pain Nurse Specialist, spoke about pain medication with the theme "We love them, we hate them, and do we need to take them." She stated that the long term use of opiates for chronic pain is relatively new. Fifteen years ago opiates were reserved for post-operative, cancer and end-of-life care. Prescribing opiates began with the mission to help people with pain. They are helping but side effects such as damage to the liver, memory, mood swings, deactivation, apathy, nausea, vomiting, sedation, overdose, increasing dosages, abuse and misuse, withdrawal, constipation are all negative factors of using opiates. She concluded that opiate use may be best limited to short term symptom management.

Pamela Narayan, Physiotherapist from India, spoke from

a perspective of limited availability to factor and therapies. Pain avoidance fostered a reluctance to re-engage in exercise. Factors such as geographical isolation, social prejudice and self-treatments meant that treating practitioners were not always aware that patients needed more help.

Kinesio tape, the new tape you see plastered all over our sports heroes is a new therapy they have been working with to increase movement and function when factor was unavailable.

Pamela concluded not to make pain the main problem but make the restoration of function the issue.

Sylvia Von Mackensen from Germany, a medical psychologist, spoke amount how mood affected our pain. She stated that 67% of adults with haemophilia had arthropathy and of these 35% suffered chronic pain. Mood affected chronic pain in 85% of those suffering. We need to target physical, emotional, social and occupational factors to improve function and increase quality of our lives. We also need to accept our pain and reduce our negative responses. Some of the barriers are that patients and doctors speak different languages and have different experience, expertise and frames of reference. Lack of sleep, feelings of helplessness and limited coping strategies are unhelpful when dealing with pain. She concluded that chronic pain in PWH cannot be cured but we can improve self-management using both pharmacological and non-pharmacological measures.

Patient and Provider: Exchanging of Perspectives

By Robyn Coleman

This session was divided into three questions in which each used a Health Care Provider and a person with a bleeding disorder to give their perspectives.

Adherence: Who is responsible - Doctor or patient?

Person with a bleeding disorder: We know what is good for us but we don't always do it. If we are on prophylaxis it can give

us a false sense of security and then we take more risks. We know our bodies better than the doctors and so we feel we can self -regulate and experiment if we think we are overtreating ourselves. We have to get up early and if we don't then it's too late, we're too tired and we leave it until tomorrow. Some days we don't even feel like treating at all! We may have trouble with vein access. We may want to keep our haemophilia to ourselves and wear long sleeve shirts.

Health Care Provider: The lack of adherence may be due to any of the following:

- Denial,
- Lack of symptoms
- Lack of communication
- Cultural issues
- Resource barriers
- Impaired family function
- Inconvenience
- Privacy and disclosure issues about HIV/HCV
- Pain of injections/ venous access

Adherence is an adult patient's choice. The health care providers need to get to know the patient's story, to assess and not assume. It is important to use an individualised approach. Pairing staff with a specific patient based on the patient's need may be a better way forward. The staff need to not take non-adherence personally.

Adherence is both the patients and the providers' responsibility.

Counselling techniques: Does talking help?

Health Care Provider: Why go to counselling? PWH often don't think anything is wrong with them and the person might have fears and pre conceived ideas about counselling. It is important to work through issues, especially if the person has HIV/HCV. Counselling can also help with feeling of isolation.

Person with a bleeding disorder: He would rather talk to friends as then he is honest. When he talks to health care professionals they just ask lots of questions and he is often not honest. He feels more comfortable sharing with other men and feels that when they have peer education they have counselling at the same time.

Sexuality and risk reduction

Patient with a bleeding disorder: HIV/HCV is not always about you but can be a partner issue also. The nurse that you grew up with is not necessarily the best person to talk about sex with. Doctors tend to lecture you about sex. The social worker is the best person to talk to and it is best if you have a male.

Health Care Provider: Social workers tend to have longevity in places such as treatment centres. Sex education is overlooked with a bleeding disorder. Expertise is needed. You need a psychosocial worker with bleeding disorder knowledge.

Parents need to be educated to teach kids about sex. These discussions need to happen at ages 14-15. Parents need to go out of the room when sex is going to be discussed with a health professional. You need to ask the right questions:, for example does dating mean going to the movies or sex? Information such as sexual activity, tattoos, drug and alcohol use also need to be discussed.

Patients are more likely to talk about sexuality with another male.

Co-Morbidities and Care Delivery

By Sarah Elliott

New approaches to hepatitis C management

Catherine Stedman, Gastroenterologist and Hepatologist at Christchurch Hospital, discussed the upcoming options for treating hepatitis C virus (HCV).

We are seeing increasing liver disease as people who have not been cured from HCV are deteriorating in health. To try and combat this there has been numerous trials of different combinations of new direct acting antiviral treatments such as telaprevir, boceprevir, ledipasvir, sofosbuvir, and in some cases these are still being trialed alongside the old drugs; Interferon and Ribavirin.

There have been some very promising results in the general population using sofosbuvir and ledipasvir (1x pill, 1x day) in combination with ribavirin. This has been trialed on around 2000 people of different genotypes and had a success rate of 97%. For PWH there has been a similar recent small trial in New Zealand of sofosbuvir with ledipasvir and ribavirin. This was trialed on 14 patients with haemophilia for 12 weeks with a success rate of 100%. There were only a few minor side effects from the ribavirin and there was no impact on their coagulation.

These are very exciting and promising results; however there are still barriers in terms of drug costs and availability.

Ageing-Related Morbidities

Evelin Mauser-Bunschoten from The Netherlands noted that as PWH are living to a similar age of the general population we are starting to see an increase in co-morbidities (other diseases on top of their bleeding disorder). This leads to new challenges, complications, and management issues which have never been seen before in this cohort, and they say these are leading to more quality of life problems (mental, emotional, physical, social, spiritual) for older PWH.

Some main/new challenges our population is facing is:

- Obesity - This can increase arthropathy and pain and limit ability to participate in exercise
- Diabetes – Same risks as for the general population
- High blood pressure – More common in PWH then general population
- Cardiovascular disease – Lower in PWH than in the general population, however, is a high risk for PWH as can cause intracranial bleeds.
- Malignancies – Same as general population, however, PWH have an increase in liver cancers (due to HCV).
- Sexuality – Can give older PWH pain and can be limited by arthropathy.

All of these need to be managed effectively and PWH need to have a GP as well as their haemophilia centre. PWH need the same regular checkups and monitoring the general population receive and need to have all medicines monitored carefully by their GP. They should also encourage comprehensive care and make sure their different specialists are communicating with one another and all on the same page in terms of managing any issues that arise.

Care Delivery through Telemedicine

Roshni Kulkarni from the USA) discussed the telehemophilia/ hemostasis comprehensive clinic.

We are starting to see an increased use of medical information done via technology. An individual can go to a local doctor and connect via technology to their specialist who may live some distance away. This can be used as a way to educate people, support people, hold 'distance clinics,' assess and diagnose people. It can be done via live video interaction, text, email, sending photos, plus many more means such as using enhancing equipment (e.g. stethoscope/telescope) that a local doctor can attach to the camera so the specialist can see and assess.

Most hospitals will already have access to some form of telemedicine service so in many cases the haematologist just needs to connect to it and learn about how to use it.

The positives are: it reduces travel time (for those who live remotely and would need to go a long way just to see their haematologist), reduces costs, improves people's access to care, alleviates a physician shortage, allows specialists knowledge to come into rural settings, means shorter hospital stays, patient satisfaction, less medication errors.

The negatives are: specialists on the other end of telemedicine can't palpate, security concerns such as firewalls, will need more IT specialists, cost to set up, and it can de-personalise relationships.

Haemophilia and its impact on employment

By Wendy Christensen

One big question all children have is whether they can be independent and what kind of work they can do as they grow up.

We need to focus on them growing healthy with a bleeding disorder and be open to different possibilities and knowing their true limitations. We need them to direct their energy into the right work path that suits each individual person.

There was a study undertaken in Germany that found that 25% of PWH do light manual labour compared to 20% worldwide and from this they concluded that the availability of treatment defines the type of work they perform. In the survey, 25% of people said it was difficult to get a job due to haemophilia, and 26% felt they would earn more money if they didn't have haemophilia.

Disclosure: When, how and who to tell about a bleeding disorder

By James Poff

The impact of a bleeding disorder on an individual and their family cannot be understated, however, once the initial diagnosis has been made and the treatment regime has been established it is mostly a case of the sufferer and the family "just getting on with it".

Where the bigger questions and concerns lie is when it comes to disclosing the disorder to a wider audience including schools, social groups, employers and future partners, to list just a few of the potential disclosure recipients.

We learnt at the WFH Conference that in some parts of the world there are potentially cultural, religious and even political restraints that can impact on an individual or their families decision on when, how and who to disclose the existence of a bleeding disorder. An example was given where in some countries having a bleeding disorder in the family affects a person's ability to find a partner, employment or even plan a sustainable successful future.

As stated by Brian O'Mahoney, as access to treatment increases so does the quality of life, education and employment opportunities allowing for the confidence of suffers to also increase when it comes to making these types of decisions.

In summary, there are no hard and fast rules when it comes to disclosing the existence of a bleeding disorder, to any particular individual, employer or even future partner.

What is important is to be armed with the "Latest & Greatest" information so you can reassure any person or group that they have nothing to fear from knowing, socialising employing or even loving a person with a bleeding disorder.

Remember in New Zealand there is a huge amount of support available - HFNZ , Outreach workers, fellow suffers, parents of suffers, HTC staff just to name a few – so when it comes to Disclosure: When, How and who to tell about a bleeding disorder – you are not alone we are all here to help and guide each other.

The Power of Resilience – Finding Balance and Navigating Resources

By Rosalie Reiri

Resilience is an attribute related to health and well-being and in particular the power of resilience is needed in everyday life in order to overcome challenges. In this session there were three speakers Richa Mohans from India spoke on 'The Power of Resilience', Edward Kuebler from Houston talked on 'Defining Normal' and Leonie Mudge from Australia discussed 'Helping Patients and their Families Identify Resources'. Due to the amount of information I have chosen to bullet point key learnings from the first and last session and spend the majority of this report on re-defining what is 'normal'.

The Power of Resilence - Richa Mohans

- People with haemophilia (PWH) show high levels of resilience.
- It is assumed that humans are wired with resilience however resilience is created and developed.
- Resilience can be modified through communication skills.
- Resilience can be role modelled.

Helping Patients/Families Identify Resources - Leonie Mudge

- The aim of the Australia New Zealand Haemophilia Social Workers and Counsellors group is to provide comprehensive care for their patients.
- Australia has 12 social workers based all over Australia who meet twice a year. The group also includes HFNZ four Outreach Workers as well.
- The Australia social workers provide many programmes such as peer support for parents.
- There was a special mention about New Zealand for the support Linda Dockrill, Southern Outreach Worker, provides regarding the Parents Empowering Parents (PEP) programme.

What is Normal - Edward Kuebler

Normal is defined as "conforming to the standard or the common type," a medical term is shown as "a natural occurrence". Normal is actually a state of mind, PWH fight to be normal as they desire to fit in so they are not seen as different. So how do we change our own mind-set to feel normal? The need to address or to re-defining 'normal' is important with people with a bleeding disorder to realise that haemophilia is normal, that limited activity is normal and the need to accept the diagnosis is normal. It is interesting to acknowledge that many people do not know they need to change or realise they are even battling with this thought, this is caused sometimes when dealing with fear. Ed shared that comfort is the way he approaches working through his own fears, he personally pinpoints what needs to change and then he starts from that place.

For more information about changing a mind-set you can use the link: www.ehow.com/how_5237982_change-mindset.html

Finding Balance

When life is busy it is easy to find yourself off balance. If you find yourself in this space it is recommended to take time out and take a 'snap shot' of your life, the analogy is to imagine a view of your life as if you were in a helicopter. By doing this you can identify the things in your life that you do that you either need to continue or stop doing to bring your life into balance. Some prompt questions to help you identify your own central point are ones like: What makes you happy? What makes you laugh? When have you felt joy? What are the things that are weighing you down? If something you do in your life does not make you happy, make the adjustments to help you find centre. Ed shared tips for finding balance and the keys to success are in time management through making time for yourself and time for your family, being creative and building a stronger support system around you.

Sometimes we can become a person that we are not and a person that we do not want to be around and in actual fact our family and close friends can think of us in this way also. So how do you want to be remembered when the dust settles? It is ok to ask for help if you need to change how you respond to situations and if you need to change how you think begin by figuring out what is most important to you and work towards achieving your goals.

Have a Plan A and B

Always have a backup plan for pretty much everything in your life and especially the BIG stuff. Stress and anxiety occurs when we are not prepared for little changes or when things need to change at last minute. Remember not to sweat the small stuff and that "perfect is the way things are and not the way you think they have to be". If you are not sure how to prepare for plan B, it starts with the question 'What if this does not work'? By doing so it allows us to think past our first plan and expectations. Ed shared an example of when he bought expensive tickets to see Cher - he caught a 3-hour plane flight, and twenty minutes before landing the plane had to turn around and go home. What could have been a night of frustration and anxiety ended up being ok as he acknowledged he was going to miss the concert, there was no way he could have made it, so luckily he was able to give the tickets to his sister who lived in the area. For tools to assist to help find balance in your life see www.mindtool.com/pages/article/newHTE93.htm

In summary resilience, balance and normality in life all starts from thought created in the mind. The most important message from this talk is the need to re-define what normal is. If you need to change your mind-set acknowledge what needs to change, make the adjustments, seek the help and strive for your perfect world of normal.

The Global Picture

WFH President's Address – Treatment for All: Forging Ahead

By Grant Hook

This plenary session was given by the WFH President Alain Weill and gave us the view from the top!

One of the main aims for the WHF is "Treatment for all". Providing affordable care and compassion has a humane element to it but there are other reasons that this will be good for all of the bleeding disorder community.

The cost of care still puts it beyond the reach for most of our community so in many places the only treatment is ice and rest. The economic status of a country denotes their capacity for medical care. WFH has the power, the means and the knowledge to expand treatment products and access to treatment throughout the world.

We have the power, the means and the knowledge to make a difference and provide Treatment for All – Alain Weill, WFH President

To achieve this we must innovate at all levels or we will stagnate and if we stagnate people in our community may die unnecessarily. The biggest issue is cost so innovation will include new treatment regimens such as longer half-life products and gene transfer therapies.

There are also economic shifts in the world with statistics showing that by 2020 developing countries will account for 33 percent of the world's gross domestic product. This means that replacement therapy use will increase in developing markets, which will in turn boost supply and create a cascade effect on pricing.

To take advantage of this we must continue to improve our data collection and to identify people with bleeding disorders in order to lobby governments for care. The 2012 WFH Global Survey included data on more than 90 percent of the world's population and identified 274,000 people with bleeding disorders in 109 countries.

WFH continue to develop initiatives to achieve these goals, including;

- Research programmes
- Training in underdeveloped countries
- Expansion of the Global Alliance for Progress (GAP) to 10 countries. As of March 31 this program has raised $4.4 million of its $5 million goal.
- The Twinning programme in which New Zealand is working with the NMO CHA in Cambodia
- Implementation of the Cornerstone initiative to assist 15 countries over the next 10 years where current standards of care are inadequate. Nigeria, Ethiopia and Nepal have been selected and we hope that our twin Cambodia will be selected to assist CHA to improve care in their country.
- WFH's ongoing work with manufacturers has made WFH the world's largest supply channel for donated haemophilia products. WHF has distributed 248 million IUs of product in 86 countries helping about 90,000 people with bleeding disorders.

While as a community we have much to be thankful for, we know exactly what hasn't yet been achieved, this includes making treatment more affordable in the world's neediest countries.

To achieve this we must innovate, educate and advocate. This encompasses innovation in diagnostics, training methods, distribution, gene therapy, longer lasting factor concentrates and more. Over the next decade WFH will concentrate on two key areas, education and data collection and analysis. This will include data on specific research questions to build an evidence base for better care.

We should be very proud to be members of such a committed and progressive organisation that represents the interests of all people equally in the worldwide bleeding disorders community.

Haemophilia management with little or no clotting factor concentrates

By Greg Jamieson

The key outtake I took from these presentations is that we are very lucky to receive the haemophilia care we do in New Zealand, particularly when you reference what happens in less developed countries.

There were four presentations and below are the key outtakes

1) First-hand experience from a person with haemophilia living in the Philippines

He is currently 50 years old and was diagnosed with mild haemophilia at the age of 8. In the early years he treated bleeds with coconut oil and herbal leaves. His key coping strategies were to have a positive attitude, get good sleep, do mild exercise, and reduce his weight.

2) Physiotherapist from Egypt

Joint pain affects 80% of people with haemophilia. Exercise is known to increase the amount of factor VIII in the general population but a question remains over whether it does the same for people with haemophilia.

His key recommendations were

- Stretch
- Think about the type of exercise you do
- Start slowly
- Warm up – Exercise - and then warm down
- No gain with pain – think about that!!
- Light weights
- RICE (Rest, Ice, Compression, Elevation)

3) Jamaican Haematologist

In Jamaica, the Government does not buy any clotting factor concentrates (CFCs). They only have what is donated through WFH or other organisation. The CFC they get is prioritised for critical patients. As such treatment is mostly done using blood products. As a result, as at 2002, 41% of patients had hepatitis C and 4% had HIV.

4) Senior lecturer from the United Kingdom

He pointed out that 75% of the CFCs are used by 15% of the global haemophilia population. In developing countries there are major issues with identifying people with haemophilia before treatment is even considered. There are four keys to setting up a protocol for haemophilia: diagnose accurately; access to expertise; advocacy, and; education.

In Our Backyards: Eliminating the Gap in Care between Developed and Developing Worlds.

by Patience Stirling

A diverse panel of speakers from developed and developing countries shared their knowledge from their backyards

Beryl Zeissink, a haemophilia nurse from Brisbane Hospital, focused her presentation on "How do they perceive the divide and how people they care for are affected by it?" This was interpreted with information on the history of the Australian Haemophilia Foundation, the years of product changes and the mishap of infected blood products. Their definition of developed is having specialists, designated staff and established haemophilia centres. The developing is limited or no resources, limited or no support staff in other areas and limited treatment.

The response from a member of the audience queried the neglect of outback communities, stating they are still developing within their own developed countries.

Marijke van den Berg from The Netherlands enlightened the audience with "What could change look like?" With three key initiatives, the Global Alliance for Progress (GAP) Programme, the Cornerstone Initiative and the WFH Research Programme, the WFH is continually working together, taking small steps to close the gap.

R. Sathyanarayanan from India explained the difficulties in his developing country. Despite the difficulties, he described how they are creating change through empowering women. There are growing active women groups who are mainly mothers of people with haemophilia (PWH), they are motivated and are breaking down the barriers for better access to treatment.

Carlos Gaitan-Fitch from Mexico clarified why the GAP exists; it's due to population, economic conditions, health policies, awareness and attitude of stakeholders. After defining each area, he then gave definitions on what it will take to make that change; sensitisation, involved in lobby and advocacy aspects, involve media and conduct education in hospitals on the disorder. His biggest challenge was to keep teaching himself that he has rights. He said where he comes from in a developing country PWH have always thought they don't have rights.

In conclusion, Jan Willem Andre de la Porte from South Africa, patron to the WFH, agreed that there are still developing areas in so many developed countries and there is still a lot of work to do but we can only achieve this with a little step at a time.

The Future of Haemophilia Care

Understanding global demand for treatment products, patient expectations and access challenges

By Linda Cole and Nigel Anderson

Chair: Mark Skinner

Topic 1: The Rise of the patient voice.

Mark Skinner

Mark gives his parents great credit for teaching him that life is manageable and he was encouraged never to feel sorry for himself. If something happened, he had to "buck up" and move on. In many ways haemophilia made him stronger and gave him the drive to excel. Living with a chronic disease taught him how to persevere and cope with adversity.

He also learned the importance of a support group that included his immediate family as well as the extended family of a haemophilia association. He was and is fortunate to live in a country that now has access to advanced haemophilia care and treatment to correct his joints. But meeting other members of the haemophilia community around the world has made him painfully aware that he is an exception, not the rule.

The power of one or two voices to make a difference is amazing. You don't need a lot of skill or expertise. What you need is patience and the heart to do it. For a young person with haemophilia, the knowledge that there's someone out there cheering them on is incredibly empowering.

Topic 2: Patient perspective – attitudes, expectations and desires for new and emerging therapies.

David Page

Many factor products are being developed now that have a longer half-life, some with 2.5 times longer half-life than the traditional product. They are being marketed with different perspectives either emphasising less frequent infusions, using half the amount of an infusion or prophylaxis treatment varying from once a week to once every ten days. The overall benefit is less frequent infusions!

However, David stressed that the goal should be less bleeds rather than less infusions!

David carried out a survey which will be available on WHF website with 217 respondents.

Unfortunately at the time of reporting this was still not available.

Topic 3: Modelling global FVIII and FIX demand for treatment of haemophilia A & B

Jeffrey Stonebraker

Problems with modelling the global demand for products include a lack of therapeutic standards across countries, uncertainty surrounding epidemiology and the scarcity of the products.

The demand for having accurate models is driven by:

1. Therapeutic demand
2. Economic development, financial constraints on countries
3. Product availability

Jeffrey analysed over a thousand health-economic indicators relative to factor use on a country by country basis. He presented how by adjusting the inputs into his model he was able to determine the key drivers for outputs. Because there is so much uncertainty around the variables modelling the global demand could take several forms.

The most sensible variable for FVIII demand was the number of bleeding episodes over the past decade. The countries with the highest FVIII use have moved closer to representing the latest therapeutic demand for FVIII in the treatment of haemophilia A.

Jeffries research has started a conversation about the future global factor demand needs for haemophilia. Still to be studied are specific demand models for developing countries and planning for production capacity.

Topic 4: The importance of patient focused outcomes data.
Randall Curtis

The Haemophilia Utilization Group Study (HUGS) surveyed several hundred people with haemophilia in the USA. The main objective of this study was to examine factors associated with utilization and costs for persons with haemophilia as well as quality of life. Several studies have been published using the results.

Slides related to this presentation will be available of WFH website – not available at time of writing.

Overall, their studies show that prophylaxis relates to:

1. Less emergency room visits
2. Less stays in hospital
3. Use of more product
4. Children have less joint issues (less pain & motion limitation)
5. Patients under thirty have an expectation factor is freely available – lacking the responsibility shown by adults

In comparison severe adults who had no prophylaxis care when young/teens have lower quality of life, debilitating joint damage, and non-working joints. This is beginning to be seen as a cause of conflict between those people with haemophilia aged over 30 in that they perceived today's children with bleeding disorders as being irresponsible about their factor usage (i.e., and playing contact sports).

Von Willebrand Disorder and Rare Bleeding Disorders

VonWillebrand's and Platelet Disorders

By Jessica Hirst

The first speaker talked about the challenges in the diagnoses of von Willebrand Disorder (vWD).

vWD is difficult to diagnose and to diagnose correctly. There are six types of vWD, Type 1, 2A , 2B, 2N, 2M and type 3. From a lab's point of view, the reason vWD is so hard to diagnose is because the molecule that has the defect in it is a very complex molecule.

The diagnosis of vWD should involve a physical examination, history, blood tests, then a repeat of blood test for confirmation, then a DDAVP trial. Then a diagnosis can be made and a management plan put in place.

The DDAVP trial test is recommended to perform because the outcome can be quite predictive of the type of vWD a person has. For example, type 2B does not respond to DDVAP in most cases.

The second speaker discussed the management of vWD. vWD has a very wide spectrum of bleeding, from very mild to very severe requiring regular prophylaxis.

There are three main treatments for vWD:

1. DDAVP (desmopressin) — DDAVP is a great treatment for some types of vWD, but it is recommended to do a test infusion of DDAVP at 0, 1, and 4 hours before a bleed or before DDAVP is needed, as there is a highly variable response to DDAVP. DDAVP should be avoided in children under 3 years, in the elderly and avoid or pre-test in type2B and with people with platelet disorders.

2. Concentrates – There is no recombinant treatment for vWD yet but there are plasma-derived FVIII concentrates which contain VWF. Inhibitors are rare, but are more common in Type 3 severe vWD. Long term prophylaxis for people with type 3 vWD is very effective to control joint bleeds.

3. Manipulating alternative routes of blood clotting – These include the use of antifibrinolytic agents, such as tranexamic acid and oestrogen progesterone drugs.

The last speaker discussed Platelet Function Disorders. Platelet disorders can be hereditary or acquired (caused from disease or medication).

Typical problems for people with Platelet Function disorder are:

- Frequent nosebleeds
- Easy bruising
- Bleeding from the gums
- Heavy or prolonged menstrual periods.
- Bleeding with childbirth
- Prolonged or excessive bleeding at time of surgery or dental work.

Bleeding symptoms can be highly variable with inherited platelet disorders such as Glanzmann's thrombasthenia, Bernard-Soulier syndrome and vWD type2B.

Treatment for platelet disorders depends on the particular type of disorder and severity of the bleeding. The recommended treatments are DDAVP, recombinant factor, tranexamic acid, hormonal therapy and platelet transfusions.

Rare Bleed Disorders (RBD)

By Joy Barrett

Much of these sessions focussed on the variety of research and BATs (Bleeding Assessment Tools) that are in use worldwide.

Although internationally many BATs are available, each has a differing standard based on its formulated research. This raises difficulties in diagnosis as clinical features are highly variable, influenced by subjective factors of patients and physicians and bleeding symptoms reported as normal. Significant advancement in commercial products for use during menstruation cycles reduce reported severity in many countries.

In general there is a lack of robust data collected and available. Small populations are effected, i.e. fewer than 1 in 1,000,000, although prevalence can be higher in areas with close family ancestry genetic links.

The role of genetic testing, where available, unfortunately leaves approximately 50% of patients with evidence of platelet disorder without a specific diagnosis. Which impacts on the individual and family concerned with issues of quality of life. But this same genetic testing is bringing characterisation of molecular defects in recognised disorders and therefore increasing knowledge.

The need for specialised care in hospital, support in the community and family, and more funding in co-ordination at teaching institutes and new treatment products at company levels has been recognised.

The WHF now have a compendium of Bleeding Assessment Tools on their website.

The whole area of Rare Bleeding Disorders and the people living with this is going to see significant developments in the coming years. But due to the low incidence within New Zealand it will inevitably feel isolating.

Factor concentrates for RBD

By Chantal Lauzon

This presentation was part of the Novel Products for Haemostasis session.

So much information centres on treatment options for haemophilia A and B. Although the demand is much smaller, there are also a number of developments in products to treat rare bleeding disorders (RBD).

The mainstay for treatment of RBDs in the past has been cryoprecipitate (cryo). Although most European countries no longer use cryo and have a few more treatment options, cryo is still used even in the USA to treat some rare factor deficiencies. In contrast, in places like Cameroon there is little to no factor concentrates at all and not even any cryo, so fresh frozen plasma (FFP) is used when available.

Table 1. Treatment options for rare factor deficiencies

FACTOR I (Congenital Fibrogen) Deficiency	• Prevalence is around 1 in a 1 million • Available treatments: FFP, cryo, concentrates. • Concentrates are the best option as others are linked to a risk of infection and allergic reaction. There are four concentrates available. • Many companies are developing new fibrogren concentrates. There is an interest because they can also be used for cardiac surgery, for example, not just treating FI deficiency. • In developing countries, there is also hope with a new filtering system for cryo (SD-F-Cryo) • There is a recombinant fibrogen produce (ProFibrix) which has very good efficacy and has recently been filed for registration in Europe.
FACTOR II Deficiency (Prothrombin Deficiency)	• Treatment options: There are no specific concentrates available but can be treated with Prothrombinex (Prothrombin complex concentrate) which includes FII, FVII and FIX, although the amounts are variable. • Usually not diagnosed in developing countries as it is very rare (only 100 cases known worldwide).
FACTOR V Deficiency	• Also called parahemophilia or Owren's disease, it affects only about 1 person in a million. • Treatment options: FFP, platelets. • There is a novel FV concentrate under development as an orphan drug. The advantage is the significant reduction in the volume that has to be administered. Efficacy looks good but it is still in Phase 1 trials. • The global situation for treatment of FV deficiency is not satisfactory so the concentrated is awaited.
FACTOR VII Deficiency	• Severe factor VII Deficiency is extremely rare—it occurs in 1 in 500,000 people. Approximately 1 out of 1000 persons is a carrier of the defective factor VII gene. However, both parents need to be carriers of the defective gene in order to pass on the disease. • Treatment options: Most developed countries have access to pdFVII and rFVIIa (NovoSeven). There is even long-acting rFVIIa in development. • Global situation is very good – there is even some rFVIIa in developing countries. • There is also a transgenic FVII under development which has been derived from rabbits. Seems to works as well as the human form (compares to NovoSeven). The first human patient has just been enrolled in a trial.
FACTOR X	• Extremely rare, known to affects only around 50 people worldwide. • Treatment options: Mostly the same as for FII - FFP and PCC. There is a concentrate Factor X P that contains factor X with FIX but it has limited availability. • BPL have also developed a high-purity FX concentrate from US-sourced plasma that undergoes three viral reduction steps. Although the pre-clinical data is good and a small clinical study is underway, it has taken 7 years to get that far. This type of lengthy development time is a big problem for the rarer factor deficiencies as are the regulatory requirements of bringing a product to market are difficulty to meet with so few patients.
FACTOR XI Deficiency (Haemophilia C)	• Affects both men and women, although women show more symptoms. In the USA, prevalence is around 1 in 100,000. In the Ashkenazi Jewish population it is thought to affect as much as 8% of the population. • Treatment options: in some countries plasma-derived FXI is available (made by BPL and LFB). • The global situation is relatively good if they can access one of the concentrates and can also use antifibrinolytics. • There is a risk of thrombosis with the use of the concentrates.
FACTOR XIII Deficiency	• Only affects one in several million people. To date only around 200 cases reported worldwide. • Treatment options: FFP, cryo (esp. in developing countries), pdFXIII (Fibrogammin P and Corifact). There are some others available in other countries. • rFXIII is available). It has been studied in adults and children. There were no thromboembolic events and only monthly prophylaxis is needed. • Overall, the global situation is good except for the problem of the price of rFXIII being 10 times that of pdFXIII

Overall, the situation for the treatment of most RBD is satisfactory for developed countries. The FV concentrate is highly awaited and having a FX concentrate will be a real plus. Having a recombinant treatment product available is ideal provided the cost is kept reasonable. Levels of both diagnosis and treatment are poor in developing countries.

Growing up with Haemophilia: Impact on the family

By Jo Brodie

Children's Perspective:

- Research shows a diagnosis of haemophilia impacts all family (child / sibling / parent)
- Children are the ones with the happy attitudes towards haemophilia
- We need to provide information, psychosocial educational resources and have a health care network
- It is important to include the whole family with all activities within the bleeding disorder world so the whole family are informed
- The priority should be the child first - bleeding disorder second
- Pay it forward - Involve youth with children's activities so younger learn from them

Sibling's Perspective:

- There are very few studies on the sibling of a person with haemophilia
- Should involve the sibling more in haemophilia care
- Sibling carriers have more to deal with then just dealing with being the sibling of someone with the bleeding disorder
- Professionals should not only focus on the person with the bleeding disorder - but the patient, siblings and parents
- Siblings encouraging siblings

Parents' Perspective:

- The parent should receive support / counselling if needed, safety and psychosocial care
- At time of diagnoses they go through a mix of emotions
- Research shows parents are most stressed when starting prophylaxis
- Having a child with a bleeding disorder is gradual learning curve - you can't learn it all in one day
- Information, knowledge, love, support (peer support) is key from professionals and other parents with children with bleeding disorders
- Good to involve yourself in peer activities like: PEP, family camps, regional camps, cafe evenings, etc.

Family Dynamics – The Ties That Bind

By Deborah Weir-Honnor

"The sibling relationship may be the longest most significant, dynamic and influential relationship brothers and sisters have" (Cicerelli 1994)

Siblings

Julia Spires from the United Kingdom presented that there are very little to no studies done on the impact that haemophilia has on siblings whether healthy or affected by haemophilia.

It is thought that siblings of those with haemophilia are often neglected, and an expectation that the healthy sibling will demonstrate adverse social behaviour, aggression, changes in academic achievement and negative attention seeking behaviour. What studies of siblings with chronic conditions such as CF, autism, diabetes and Downs syndrome have found, is that they show strong positive characteristic, kindness, empathy, awareness of the needs of family and others and an emotional maturity.

Adolescents and Parents

Jessica Li from China gave an overview of issues facing adolescents.

Haemophilia in Adolescence:

Adolescence is the most wonderful period of our life, but as we all know it is also a dangerous period which influences our personality. The most important thing for adolescent children is to prove they are no longer children. All adolescence children want to show their personality and that they're as independent and equal as grown-ups.

Typically rebellious and struggling with their physical growth young people with haemophilia who receive adequate treatment hope to get rid of the medical intervention and cautious parents and hope to become more independent. Whilst those whom have little treatment begin to doubt they have anything wrong with them and further conflicts occur.

Behaviours in Adolescence:

Adolescents with haemophilia often have low self-esteem, poor self-image, and are solitary. They often attribute all of the reasons that they could not grow up like other children to haemophilia, and they face life with a negative approach. Parents are often willing to provide the adolescent with haemophilia with everything including material things that their child wants as a kind of compensation to try and boost their child's self-esteem. Then the children come to expect these compensations such as branded clothes, high-end mobile phones and computers. These material things improve their confidence and feelings only in the short term, soon the children are addicted to another world and escape from the real world.

Source of Adolescent Problems:

Parents often spoil and overprotect a child with haemophilia. They make self-sacrifices and give everything unconditionally. When children with haemophilia are adolescents many parents find their children's condition has not improved, but deteriorate in spite of previous efforts. They begin to feel discouraged. In addition to these, if adolescents are insubordinate the parents may feel more annoyed and ignore their children. Sometimes families break down and divorce occurs at this stage.

Pressure:

For a long time haemophilia has been a pressure faced by children and parents. Parents are faced with the lifelong pressure of caring for their child with haemophilia. In a long term stressful situation as this, they may struggle to adapt to adolescent changes. The children are faced with suffering the disease and self-care. In adolescence, children always arbitrarily make some wrong decisions', often having severe consequences' with their haemophilia.

Communication:

Communication is the key between parents and children. They must be respectful of each other. Listening carefully is actually the most important part of communication with your adolescent. Listening doesn't mean not giving feedback. It is important to grasp the details of communication such as: take the right time, create opportunity, express understanding and love, talk less, carefully use criticism, allow tantrums and crying, sincerely praise, respect privacy and keep secrets.

Directions after Establishing Trust Relationships:

As parents, watch out for bad habits such as smoking and drinking in your adolescent with haemophilia. Discuss the value of life. Learn to face the disease in the right frame of mind. The adolescent often has built a new relationship with their parents, and now makes better contact with society. They have a better willingness to work and study, and begin to love others.

The Cost of Poor Oral Health in children with Bleeding Disorders

By Lyn Steele

This session discussed the management of early tooth decay (caries) in children and adolescents. It causes pain, infection, disturbed sleep, affects school performance and self-confidence.

Tooth decay is very common but the management of it in children with bleeding disorders can be hard. Tooth decay needs four ingredients to occur – teeth, bacteria, food and time. Take away any one of these and you don't get decay.

Many years ago tooth decay was managed by drill and fill. Now we have early interventions and prevention which treats the disease not the effects. One successful prevention method is fluoride varnish from six months of age, which is effective but expensive as are fissure sealants. Another is fluoride toothpaste brushed on the teeth. When the teeth are brushed, the mouth should not be rinsed as the fluoride should stay on the teeth and work, not be washed off to go down the drain.

Dentists can look for guidance for treating people with bleeding disorders in a number of publications but they always very user friendly and evidence based. The WFH dental committee is working on gathering input for positive new guidelines and training new staff which could be a lengthy process.

Women

Sharing information with carriers and those who care for them

By Pip McKay

The 'We should talk: Sharing information with carriers and those who care for them' session was an eye opener for me. Being newish to the world of haemophilia, my partner Karl has severe haemophilia and 2-year old daughter is a carrier, I knew the basics. I didn't understand the depth of symptomatic carriers.

This session discussed the facts about how 30% of carrier females are diagnosed as being symptomatic. It covered in depth how females are diagnosed as symptomatic with a special test called a BAT (bleeding assessment tool). However, I also learnt this test doesn't always evaluate the bleeding correctly as other outside influences can affect factor levels. Some of these influences may include stress, medication (for another ongoing problem), being pregnant or by simply being unwell. This means that that factor levels are not always an ideal measurement for symptomatic carriers.

Another part of this session went over carriers in developing countries. Because testing is very expensive in these countries a lot of women lead their lives never being diagnosed. This includes Megan Adediran from Nigeria who only found out that she was a carrier when her first son was born and diagnosed as having severe haemophilia. Megan told how families with haemophilia in developing countries can face stigmatization and the shame of being a carrier and passing this through their families. These women usually have very little support. The stigmatization is so large it affects the women's jobs and marriages. She described how some men have felt they have been tricked into marriage once children are born and have been diagnosed with haemophilia. She also explained how many women who carry the haemophilia gene are lost at childbirth from excessive bleeding as many of them have not been diagnosed.

The third part of this session was about a group called "Code Rouge" in Canada. Their first goal is to ensure women who have inherited bleeding disorders are correctly diagnosed. Another major role of Code Rouge is to increase understanding as years ago many people were of the opinion that woman don't bleed. With advancements in medicine it is now understood that woman do bleed and can bleed as excessively as men. This group also offers support to its members who are new to the world of bleeding disorders. They aim to increase the number of care programmes available to women with bleeding disorders in Canada.

Sexuality in women

By Kahurangi Carter

Sexuality in women is not the easiest topic to broach and when paired with a bleeding disorder (Yes, girls have bleeding disorders too!), treading gently might seem the best way forward. Unfortunately, the sensitive nature of heavy menstrual bleeding or abnormal menstrual bleeding (AMB) as health professionals now call it means we don't talk about it enough. This can leave girls and women to suffer in silence without reaching out for support from their significant other.

The good news for sufferers is there are effective means of treatment to help with AMB including the contraceptive pill, Mirena implant and tranexamic acid. It is important for women to overcome their embarrassment and seek the help of health professionals.

AMB is not the only thing that contributes to a women's intimacy challenges, easy bruising and nose and gum bleeds don't exactly leave you feeling sexy. Improving quality of life; physical, social and emotional well-being can be achieved with education and support.

Women can find it hard to talk about this subject with each other let alone their partner. Having the support and tools to decrease the stigma and embarrassment of excessive bleeding and the effects that come with it, will lead to open, honest, healthy relationships.

You may never have realised how you or your partners AMB has held you, your family and/or your relationship back or maybe you have and you had just resigned yourself to a life of uncomfortable situations. Remember you are entitled to the best life you can have and with the help of your doctor and the support of HFNZ, your outreach worker and your partner and family, it can become your reality. An international support group called Mygirlsblood is a network on Facebook who are here for you.

Building Capacity

Leadership Development Strategies: Am I Ready To "Carry The Flag"?

By Tuatahi Pene

Leadership Development Strategies: Am I Ready To "Carry The Flag"? was presented by four speakers each showing their different perspectives on the above topic. Mike Skinner showed the importance of leadership. Ed Kuebler covered leadership development in the bleeding disorder community. Pedrag Mikov gave youth experiences from a male perspective and Evelyn Grimberg gave the female perspective on youth experiences. My take on this report is going to focus largely on the presentation of Mike Skinner showing the importance of leadership.

Mike's talk on the importance of leadership showed how people can be groomed into a position of leadership from within an organisation. The focus of grooming should lie on the current leader of an organisation or the example he used in his presentation was the CEO of a large corporation into first to think about succession when stepping into the role of CEO.

The qualities of a leader are the first thing that needs to be identified before having someone chosen for the role. The next step is to distinguish from the peon group of people who are the ones that fit the role - keeping in mind there could be more than one. Follow a process of elimination by providing the distinguished people certain tasks which will hopefully let the leadership qualities flow, this will also ascertain who are more qualified for the role.

The process above is a broken down structure that was presented by Mike that shows not all leaders are born but are sometimes made.

Medical and Lay Collaboration

By Theresa Stevens

Pam Wilton a mother of a child with Haemophilia from Canada opened her part of this presentation with the following statement 'consumers blend well' . . . . she continued by saying how important it was to have a Comprehensive Care Team (CCT) as this allowed for constructive cooperation. The team should consist of at least two people with bleeding disorders, one parent, physiotherapist, social worker, nurse, medical professionals including a hospital administrator.

The Comprehensive Care team allows not only increased communication between the CCT and the consumer but also an increased understanding and awareness including panning, implementation and the evaluation of care which ensures optimal health care and prevents complications. It provides decision support, enhances programmes and promotes health and wellbeing of those living with bleeding disorders. The benefits of a CCT include an effective cost solution, efficient planning which ensures needs are being met. Benefits include an increase in understanding and awareness of issues for patients and staff which result in a respectful and effective partnership. Patients are able to provide input into policy and into programmes which assists in facilitating changes.

Of course with all good things there are challenges! Time, travel, confidentiality especially in small town areas, when you are trying to maintain confidentiality in relation to a particular issue. Bleeding disorders are rare and therefore there can be lots of competing. Often authority is vague and certain things may not be implemented.

Pam then asked how do we know it works – we don't! Some things make good sense and collaboration is powerful.

Successful Partnership – Stronger Together

Basically it was to strive for positive collaboration and cooperation between lay and medical people. These issues are important and deal with ethical and human rights. They include but are not limited to preventing illness and alleviating disability, strategic planning and prioritising. Improving care is our responsibility. Start by setting goals, plan, resource, implement, evaluate and improve if need be.

Share Responsibility – Equality in Decision Making

This topic discussed the standards, advocacy and clinical and technical expertise. Critical elements of a winning combination include equal and mutual respect, transparency in relation to communication, sharing of information, increase trust and never compromising the NMO integrity. The third point is based on results being real action orientated; setting mutual targets after you identify and acquire resources prior to executing your plans before you evaluate the results.

Responsibilities are defined as an ethical obligation to each other which is relevant and appropriate. Compliment as diversity is an asses, input to challenges and downstream impact and identification is the key! Weaknesses are the volunteers with zero financial muscle and often differing visions. Volunteers can have unequal commitment and lack NMO involvement. Critical Partnership includes leadership camps, PEP training, established Nursing training and a web register. There are formal meetings with national strategic planning. Frank Schnabel's common goal and objective was 'treatment for all'. To improve care, Nelson Mandela stated 'it is in our hands'

Beyond Registries: Integrated Information Systems

By Michael Ho

Integrated information systems enhance data collection by making it more convenient for patients which improve compliance. This in turn provides clinicians with a holistic picture and timely information of patients' health, ensuring better outcomes and optimal use of resources.

Traditional patient registries are hard to manage and do not provide clinicians easy access for decision support at the point of care. Personal

Health Record (PHR) systems go beyond these registries by empowering users to maintain and manage their health information in a private, secure and confidential environment and with clinicians' remote access providing better decision support.

Ireland, Australia and USA shared their experiences in implementing their PHR systems, we learnt about the benefits and the pitfalls in how they rolled out their systems.

The shortcomings of the traditional systems are:

- Disjointed, mostly paper based, no real time or timely access of data
- No remote access by clinicians or HTCs (Haemophilia Treatment Centres)
- No "Decision Support" at point of care in home or hospital
- i.e. Recalls, Treatment of choice, Specific bleed, Reminders
- No real time tracking of medication with immediate recall capacity
- The Hepatitis C infection due to incomplete product recall in the 90s was a wake-up call
- No monitoring of adherence and bleed records, it is estimated between 20 to 25% at best compliance with paper based infusion records.

Positive outcomes of their automated systems

The key element of their integrated and automated systems is a unique Barcode on each product, which allows for accurate tracking of product from ordering, delivery and usage, eliminating paper trails and improving accuracy by removing duplicate data entry. It enhances safety by alerting users of expiring or recalled product when they scan the barcode at infusion.

These systems also benefit the industry by reducing counterfeiting (6 to 10% medicines worldwide), providing audit trail and lowering inventory holding costs (6% in year 1). They have recorded substantial improvement in the year following the roll out, product wastage reduced from €0,216 to zero and documentation errors reduced from 12% to zero. Over €5 million worth of medication stock has been removed from the supply chain. A Mock Recall exercise identified the location of all (100%) Medication within 10 minutes

By making it easy for patients to maintain infusion records, it improves compliance. They simply scan the barcode at infusion, without typing any product info and the usage stats are fed back to their HTC in real time. Bleeds are easily reported by clicking on the part of the body where the bleed has occurred and can be done in under a minute. Their HTCs are immediately alerted with the bleed report, and patients are also reminded to contact their HTC. They can obtain reports on all infusions, whether at home or given at hospital, in one place, providing them a complete picture of their usage history.

Haemophilia is a rare and chronic disease, adverse events are even rarer, outcomes are observed through research over the long term. The American Thrombosis and Haemostasis Network (ATHN) provide a platform to pool research data to help identify trends and patterns.

Data is collected from 137 HTCs across the USA. Identifiable information is entered by HTC staff into the ATHN Clinical Manager, it is accessible only to the HTC. To protect confidentiality, individuals are assigned a unique, computer generated code. All data going to the ATHN/Study sponsors has the patient ID removed.

Clinical plus Research Repository (data and specimens), provides these benefits to patients:

- Better coordination of care with provider
- Early trends signalled by health indicators
- Ability to contribute to the future through research

Some of the lessons learnt from the implementation of their integrated systems:

- Using standard barcoding (GS1 and ICD) underpins the system
- Minimise functional specs to reduce complexity and cost
- Variety of 3rd parties allow the choice of best of breeds for each part of the system
- Smartphone App is preferred by patients over dedicated devices
- Need for agile reporting system
- Design led by Clinician and Patients increase acceptance and effectiveness
- Not linked to any pharmaceutical companies provide independency
- Information at point of care and remote access hugely beneficial to clinicians
- Financial return on investment facilitates on going investment (reduce wastage and inventory cost)

What do patients think?	Satisfaction
Convenience of new service	100%
Privacy and confidentiality	99%
Overall services satisfaction	89% very satisfied 10% satisfied 1% dissatisfied

Australia's MyADBR system cost around $800K to develop. Both Ireland and Australia said they are happy to share their experiences, expertises and resources.

For more information:

- Australian Bleeding Disorders Registry (ABDR)
  http://blood.gov.au/myabdr
- Haemophilia Treatment Traceability http://www.gs1ie.org/Healthcare/Resources/Case-Studies/Haemophilia-Treatment-Traceability.html
- American Thrombosis and Hemostasis Network (ATHN)
  My Life, Our Future: Genotyping for Progress in Hemophilia - http://www.athn.org/content/my-life-our-future

Assessment of Outcomes in Haemophilia

Three perspectives - the physician, the financial manager and the patient

By Colleen McKay

Outcome assessment tools are useful to all involved in the management of haemophilia. The perspective of the physician, financial manager and patient, and how these tools are best utilised, were presented at the medical session "Assessment of Outcomes in Haemophilia'.

Physician Perspective:

The physician's perspective was presented by Victor Blanchette, Medical Director at the Hospital for Sick Kids in Toronto. Dr Blanchette explained that one of the biggest challenges in haemophilia care is joint health. Measurement tools should assess the effect on the body's structure and function, the patient's ability to perform activities and the patient's participation in life events. Good outcome assessment tools should be reliable, valid and sensitive to change. Culturally specific versions in a variety of languages should be used to enable the tools to be used in other countries and for the outcomes to give comparisons within a global perspective.

The WFH recognised the need for a standardised scoring method many years ago. One early tool was the 'Gilbert' joint score. For each of the six joints (bilateral ankles, knees and elbows) a number is assigned. The limitation of the Gilbert joint score is that it doesn't apply to young children. However, the Haemophilia Joint Health Score (HJHS) which also assesses the ankles, knees, and elbows, is validated in the paediatric setting.

Blanchette said that if a tool is to be globally useful, it should be translated into numerous languages and be culturally sensitive. He referenced the Haemophilia Activities List (HAL), which does have paediatric version, but also has culturally inappropriate questions that would limit its use in some countries / cultures.

Collaboration with Haemophilia Societies who can explain the usefulness of assessments and their relevance in publications such as their newsletter, on their website and at camps, workshops and conferences is recommended as this will increase patient uptake.

Funder Perspective:

Leigh McJames, General Manager of the National Blood Authority in Australia, spoke from a 'funders' perspective. It is well accepted that within the current economic climate that the health dollar has to go further and further, that this is a shared responsibility. McJames claims this can only be done in partnership with the patient and clinicians.

In Australia, states and territories contribute approximately 37 per cent of funding to the Blood Authority and the Commonwealth contributes 63 per cent. The National Blood Authority has a framework based on five quadrants – comprehensive care, supply of product, information and data systems, knowledge development / practice improvement and stakeholder partnership collaboration.

It is a simple statement of fact that the funding is much higher for haemophilia than in other illness areas. The Australian system delivers excellent comparative value for money, but it remains essential to demonstrate that we are delivering the best possible outcome and are fiscally responsible.

The treatment of haemophilia is entering a new era – the longer acting products will introduce another aspect; different and better treatment products. Funders will look very closely at the health economics of the new haemophilia treatment products that are entering the market. Health Ministries are not interested in paying more and more money for the sake of convenience. However, they will consider improved clinical outcomes, improved Quality of Life for people with haemophilia, less bleeds, less sub-clinical bleeding, higher trough levels, less frequency of infusions for children, and the better health of veins.

Patient Perpective:

Brian O'Mahoney, CEO of the Irish Haemophilia Society, provided the patient perspective of the topic. 'Treatment works and it transforms lives, but saying this is not enough. We must prove it using outcome data and this requires a solid case based on economics' said O'Mahoney. It is important that non clinical outcome data be collected by Haemophilia Society Patient Groups. Alongside the collection of hard data, it is also important to collect soft data and relevant photographs, the 'human element', to go alongside; this is especially important for presentation to funders - the results can also be used in the Annual Report to justify funding of the service.

Surveys must be short, not too complex, and easy to use, as well as being seen to be important. It is recommended to use a range of collection methods in order to reach the maximum audience and seek maximum uptake - phone surveys (smart phones make this easy), mail surveys and web-based surveys.

It is important to gather data; it is important to know why you are collecting the data, where you want to use the data, and how to use the data once collected. It is important too important for members to understand the rationale for the collection of data; this will increase survey uptake.

Once collected outcome data can be used:

- To prove efficacy of treatment
- To justify product consumption
- To advocate for patients
- To secure funding for the service

Surveys have enabled the Irish Haemophilia Society to collect a full set of data for their Hepatitis C Database; this data has enabled the Society to successfully lobby government and the Minister of Health for the funding of new Hepatitis C treatments.

Healthcare is undergoing a revolution. Governments want more evidence that resources are well managed. The patient perspective is different and must be heard in any debate on access to treatment; the values and the goals of patients are important. There is shared responsibility and it is important that savings are directed into services for those with bleeding disorders - Physiotherapy, Haemophilia Treatment Centres and more Clinical Haematologists - to allow for better monitoring. Patients have a responsibility to participate to improve clinical outcomes within an economic envelope.

GLOBAL NMO TRAINING

By Courtney Stevens

The opportunity for me to attend the Global NMO Training was one of the most rewarding, and at times humbling, experiences of my entire life. Initially, there were nerves about not knowing anyone else, but they soon disappeared once I arrived and started getting to know everyone else who was there. The chance to meet people with haemophilia and other bleeding disorders from literally all over the world was truly amazing.

NMO stands for national member organisation, meaning the haemophilia organisation in a country that is a member of the WFH. NMO training was run in two ways – each delegate was separated into four groups according to their country (Emerging 1, Emerging 2, Spanish and Established) which made the facilitation of the workshops more specific to the needs of New Zealand and the other countries in relation to their status as a country. And secondly, there were plenary sessions on all three of the days as well.

The training began with a plenary which included a welcoming, group dynamic activity and some of the NMOs sharing of their best practices.

Then in the afternoon we were split into the four groups and had workshops – ours was about Youth Leadership Training led by Suzanne O'Callaghan (Australia). During this initial workshop, we completed exercises about how we can engage youth within our NMOs and ways to do this. New Zealand and the United States had previously been chosen to submit presentations about our youth leadership programmes which were given at this time as well.

The second day brought a workshop about pharmaceutical relations which focused on ways to gain pharmaceutical funding and the importance of having a written policy about this. Brian O'Mahony (Ireland) was the facilitator for this and also discussed some of the developments of long-lasting factors and other developments in haemophilia care. In the afternoon, we attended a plenary about women with bleeding disorders (a first for this type of event), and had a session about clinical research in haemophilia.

On the last day we had a workshop about data and the economics of bleeding disorder care run by Declan Noone (Ireland) which provided some insight into the way governments make funding decisions purely based on cost and we did a group exercise that looked at alternate treatments, each with their own risks and had to decide whether or not it was a viable option. The plenary in the afternoon focused on an update from WFH, a question and answer session, congress tips and an evaluation period.

During two of the lunchtime sessions of NMO training I also attended a youth meeting, in which all youth were invited to attend. At the first meeting we all introduced ourselves to the group and shared some experiences of what we do within our own NMOs and at the second meeting we came up with ideas about how to engage and involve other youth members of our NMOs and what WFH can do to help with this.

The final evening was finished off with a banquet dinner in which we all got to know each other just a little bit better on the dance floor. By this time, most of us had met already and had a great time. Overall, I would definitely say that attending NMO training, as well as Congress was one of the best experiences of my life so far. I made so many new friends and learned so much about haemophilia care all over the world, making me truly appreciate and understand how lucky we are in New Zealand to have treatments readily available from the government. I have returned home armed with so much new information and so many new ideas about how to engage youth within HFNZ.

Congress Experiences

Here a few of the HFNZ delegates share their thoughts and experience of the WFH World Congress in Melbourne.

My Experience

Tena koutou katoa, ko Carol Reddie taku ingoa.

Hi all, my name is Carol Reddie...

I was lucky enough to be able to attend the WHF World Congress 2014 in May along with other HFNZ members. I was asked to write about and feedback from the plenary session set for me. I am sorry to say that I was not able to decipher all the technical stuff so I took another option offered to me and that was to give my overall view of the Congress.

The location of the venue was ideal and was well sign posted inside and outside the venue. The sessions were well spaced and times were manageable by most attendees. There were a lot of miles covered over the five days, to, from and at the venue. The exhibitors were extremely welcoming and the exhibits were easy on the eye, revealing their intention such as the interactive sight and sounds.

Now for the sessions; in my opinion the congress was tailored to all health professionals with the view to creating more motivation to shift their ideas around the world and to encourage funding of all levels... As a Haemophilia carrier and mother of two adult sons with Haemophilia A, my understanding of the new and improved treatments is growing stronger for me in as much as I will be able to pass this new information on especially the new products yet to get approval, ie.. longer half life?...watch this space.

Youth Perspective

By Dylan Christensen

Traveling to Melbourne as a youth was a big eye opener meeting lots of other youth from around the world and spending time talking to them about different things they face in their home town. We are so lucky in New Zealand with all the help out there to make our lives as comfort as possible.

There were a lot of different workshops to attend; some were over our heads and others we could relate to easily. I enjoyed listening to patients and I things I need to think about in the future with starting a family. The workshop I enjoyed the most was about how they take the younger children on prophylaxis to the lab and do blood tests with them before factor and then again after, and show them the difference as most of them have not had bleeds in their life and don't understand why they need to factor each week. As a result the children were able to managed there life better.

Thank you so much for giving me the opportunity to attend World Congest as a youth delegate.

Cost versus Benefit

By John Tuck

Severe Haemophilia A with Inhibitors

I had a right wrist bleed (old target Joint) on the Tuesday of the World Congress and I did not attend the seminar I was to write up but I thought of another topic.

As an explanation to this article, previously I had been on National Council. During my time on National Council the subject of cost of sending Staff, National Council, Branch Committee or Members to World Congress was always a major issue.

I wish to thank the members on National Council for funding myself and all who attended, the most ever sent to a World Congress. I had booked and paid for "my big O.E. to Europe" 2 ½ years ago and attended the Paris Congress so this is my second event.

Cost v Benefit for sending anyone from the groups above.

National Council has to budget for this event approximately 2 years in advance and agree on the sum budgeted. But most importantly this money has to be fundraised for through a variety of means. Bearing in mind that every dollar spent on international travel and World Congress is a dollars not spent on members in New Zealand, this raises the questions:

1. Is it acceptable for donated monies to be spent on international travel and World Congress?

2. Would donators be happy with apportion of their monies being used for such events?

Personally, I believe HFNZ can prove and justify this.

Could HFNZ provide an event to equal or inspire our members?? In short – I believe NO.

At the Melbourne World Congress there was 68 Sessions over 3 and half days plus nine lunchtime Industry Symposias, some of which we (non-professionals) could not attend. The range of topics and detail at the sessions was amazing. Some sessions had "H" Men, Partners, Parents, Medical Professionals and Scientists as speakers. As you can imagine the Medical Professionals and Scientists technical descriptions, sentences, paragraphs and in fact whole Sessions was "Mind Blowing". Other speakers spoke in a more understandable language!!!! I believe this empowered all Members and Employees.

The other important aspect of World Congress is the opportunity to interact with people associated with "H" from around the World. For instance, I spoke to two leading Doctors associated with Inhibitors, a Social Worker from America regarding the issue of Patent Confidentiality and how it is hindering Support Networks. Fellow "H" + Inhibitor guys from "The First World" about the products and dosage amounts they use. I also spoke to a guy from "The Third World", he said he only gets treatment when / if Drug Companies Donate Expired product!!!!! To hear stories from Deeply Religious Countries where Haemophilia or in fact The Haemophiliac is hidden so for instance, his Sister may have a chance of Marriage!!!! This was sad and astonishing.

In summary:

1. Is it acceptable for Donated Monies to be spent on International Travel and World Congress?

2. Would Donators be happy with apportion of their monies being used for such events?

3. Bearing in mind every dollar spent on International Travel and World Congress is a dollars not spent on members in New Zealand.

4. I saw our Outreach Workers, filled with knowledge, inspired and able to do more for our Members. It was really good to see all of our Outreach Workers at this World Congress. I understand this was the first time all have attended the same event.

5. We, the people of the "H" Community returned home with a huge knowledge about research and future products. Plus the latest ideas associated to Bleeding Disorders currently.

6. I personally will be raising questions with my Specialists about the latest techniques and treatments relating to Inhibitors.

7. I want to advocate, to attempt to eradicate my Inhibitors; However, I am now very aware how LUCKY we are to live in New Zealand in comparison to living in a Third World Country.

Once again I wish to thank HFNZ for allowing me to attend the World Congress in Melbourne.

N.B. The above thoughts and words are solely my own and I have not been coerced to discuss this topic.

World Haemophilia Federation General Assembly

By Richard Scott

After the Congress presentations finish on Thursday there is a meeting of all the member countries all day the next day. There were 83 full member nations represented at the WFH General Assembly.

Each country can have one delegate and one alternate who can vote if the delegate is not able to. The WFH board all sit up on the stage on a long table and the meeting is chaired from there. The Assembly is set up like a United Nations meeting with flags and names cards for each country and the hall is set out in alphabetical order. So New Zealand is between the Netherlands and Nigeria.

The chairperson is very strict and you only get to speak for 2 minutess if you want to say anything. There were several items of business as the WFH had to make technical changes to its' constitution to ensure the WFH met the conditions of new Charities Act in Canada, where the WFH office is based. This was very procedural and went through unanimously.

Some new nations (Bolivia, Cote D'Ivoire and Ethiopia) were welcomed as member nations. This was very exciting for these countries and it was the highlight of the meeting for me to see Cambodia also welcomed as a national member organisation.

This was followed by elections for the various positions on the WFH board. Some positions had only one candidate so we just voted by raising our green "yes" voting cards. The positions that were contested allowed for 2 minutes speeches for each candidate. Some of the speeches were very entertaining and inspiring. A secret ballot was held for each of the lay member positions.

There were several candidates and only two positions. Deon York, HFNZ President was one of the candidates nominated for re-election as a lay member on the WFH Board, after having served in that position for the last 4 years. Deon spoke well in his speech and explained all the different things he was involved in for WFH over the last few years.

Unfortunately he wasn't successful in gaining re-election, but it does look likely that WFH will continue to ask him to work on projects.

The other main vote was in regard to World Congress in 2020 which will be held in Kuala Lumpur Malaysia. The votes were 36 for Kuala Lumpur, Malaysia and 24 for Montreal Canada. The Montreal presentation was very well done but it seems Malaysia may be a bit easier to get to for more of the NMO nations, New Zealand included. Congratulations to Kuala Lumpur.

WRAPPING IT UP

The Congress presented so much information on so many areas. The very last presentation of the meeting did an excellent job of bringing a lot of what we had heard together and leaving everyone feeling hopeful for what is to come. (N.B. A report on this session is also included with the Medical Treatment and Advances reports)

Past, Present and Future of Prophylaxis (a future with novel therapies)

by Carl and Colleen McKay.

Mike Makris presented the final Plenary of the 2014 World Congress – we both really enjoyed this session. Mike Makris very cleverly brought a lot of what was presented together into this one last session.

This session was also like a trip down memory lane for us as he spoke about the past, which very much resembled our haemophilia journey – we remember the early Factor Concentrates (the huge volumes of Factor of the early years), we remember the relief that we felt with the introduction of viral inactivation, we remember the independence that we felt once on Home Therapy, we remember the excitement of Recombinant Factor Products, and now we are excited for the future of haemophilia treatment with novel new therapies, longer acting products and maybe at last the long talked about gene therapy.

The World Federation of Hemophilia (WFH) was established in 1963 by Frank Schnabel, a Montreal businessman born with severe haemophilia A. His vision, as he stated, was to improve treatment and care for "the hundreds of thousands of haemophiliacs" worldwide through a new international organization. At that time, over 50 years ago, there was no treatment for haemophilia. The life of a person with haemophilia was characterised by painful bleeds and crippling arthritis, and many people with haemophilia died at a young age.

1964 was a great year. Haemophilia pioneer Judith Graham Poole discovered cryoprecipitate which meant, for the first time, that those with haemophilia could receive treatment to control bleeding. This discovery was followed in the 1970s and 1980s by the development of plasma-derived factor concentrates. However, treatment for haemophilia was still on demand, and haemophilia was considered to be 'a terrible disease'.

Recombinant factor became available in the 1990s – 2000s. However, these products all have the same pharmacokinetics. All of these advances have transformed the lives of persons with haemophilia – particularly for those in developed and wealthier countries that have been able to take full advantage of all of these advances.

And now over the next few years we will gain a new repertoire of longer acting factor concentrates.

We currently have effective prophylaxis which reduces bleeding and preserves joints. Other benefits of prophylaxis include less need for orthopaedic surgeries, less need for hospitalisations, less time lost from school / work, and better quality of life.

Although prophylaxis works there are problems with prophylaxis with current factor concentrates:

- Cost – Factor concentrates are very expensive. Much of the world cannot afford factor or prophylaxis. This creates unfairness in the treatment haemophilia on a global scale.
- Venous access - These medications need to be infused in a vein which requires skill and healthy veins. It can be especially difficult in the very young and the very old.
- Adherence – This is a problem everywhere with current 'short acting' concentrates. Being adherent is defined as taking at least 80% of prescribed doses.

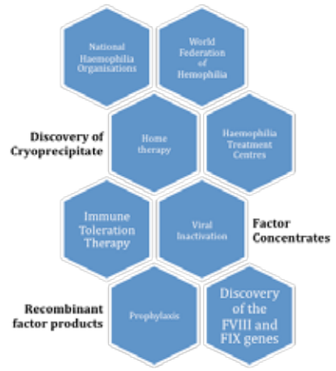

Figure 1. Progress in Haemophilia Care during the latter part of the 20th Century

Even when it is available, it is well reported that adherence to prophylaxis with current 'short acting' concentrates is not great. Why? There are a number of barriers to patients' adherence to prophylaxis:

- Cost and availability of clotting factors
- Time and Inconvenience of infusions
- Difficulties with venous access
- Perceived need for prophylaxis by patient and family

Longer acting factor concentrates could help greatly overcome some of these barriers. How will these longer acting concentrates impact on prophylaxis? There could be fewer infusions and higher trough levels of factor in the blood. The benefits of each were examined with the perspective of what goal is trying to be achieved with the use of longer-acting factor.

Fewer Infusions:

If the goal of using a longer-acting factor concentrate is to reduce the number of infusions while still maintaining a trough factor level over 1% then:

- A haemophilia B patient treating with long acting Factor IX every 10 – 21 days could expect to reduce infusions from 104 infusions per year to 18 – 36 infusions per year.
- A haemophilia A patient treating with long acting Factor VIII twice weekly could expect to reduce infusions from 182 infusions per year down to 104 infusions per year.

The benefits of having fewer infusions include:

- Fewer clinic visits or outreach nurse visits when starting the patient on prophylaxis. This might lead to earlier starting of prophylaxis.
- Less need for central venous lines, such as portacaths; this would generate cost savings and reduce the risk of infections.
- Would allow for more convenient dosing times. There will be less need to administer factor in the morning, leading to better adherence.
- Increased uptake of prophylaxis among patients not currently on prophylaxis, for example those with moderate haemophilia.
- Overall better bleed protection.

Higher Trough levels

If the goal of using a longer-acting factor is higher trough levels, this can be achieved through more frequent dosing of long-acting factor (i.e., once or twice weekly) which will take the patient from someone presenting with a normal factor level in the peak period to someone presenting with a mild factor level in the trough period.

The benefits of higher trough levels are that they allow for a higher level of activity. This regimen would give good cover and good joint protection to those who are extremely active, e.g., children. However, it is still necessary for patients to have good venous access for a regimen of treatment such as this.

Conclusion

Longer acting factor concentrates represent a major advance in the management of haemophilia. Their introduction will have a big impact on prophylaxis as we know it. There will be much more individualisation of prophylaxis with these longer acting concentrates, as the dosing interval can be tailored to an individual person's bleeding tendency and half-life.

There will be a great need for well-designed, prospective, multi-centred studies that look closely at patient outcomes, patient quality of life and the cost-benefit of these products. There must also remain an ongoing scrutiny to look for potential safety issues with these molecules.

These molecules are likely to be only stepping stones with the future development of:

a) Manufactured products with even longer half-life

b) These products being partnered with other therapies eg: anti TFPI (for more effective prophylaxis)

c) Subcutaneous or even oral delivery of such products.

And gene therapy is getting closer. How will it impact on the use of these products? There are exciting times ahead for the treatment of those with bleeding disorders.

Dates to Note

11-13 July 2014
National Inhibitors Workshop
Auckland

8-10 August 2014
Youth Leadership Weekend
Christchurch

13 September 2014
HFNZ National Annual General Meeting
Auckland

23-26 January 2015
National Families Camp
Camp Keswick, Rotorua

More details on all events are available from your local Outreach Worker.

Haemophilia Foundation of New Zealand Inc. HFNZ

NATIONAL ANNUAL GENERAL MEETING

JOIN HFNZ IN AUCKLAND ON SATURDAY, 13 SEPTEMBER 2014

FOR THE ANNUAL REVIEW OF HFNZ'S GOALS AND ACHIEVEMENTS FOR 2013/2014

TIME AND VENUE TO BE CONFIRMED.

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

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