Haemophilia Foundation of New Zealand Inc.
HFNZ

# bloodline

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Newsletter of the Haemophilia Foundation of New Zealand Inc

## Adult Wellness Weekend a Winner!

## 2010 WFH Hemophilia World Congress

*Buenos Aires*

bloodline supplement

**September 2010**
*Volume 38 I Number 3*

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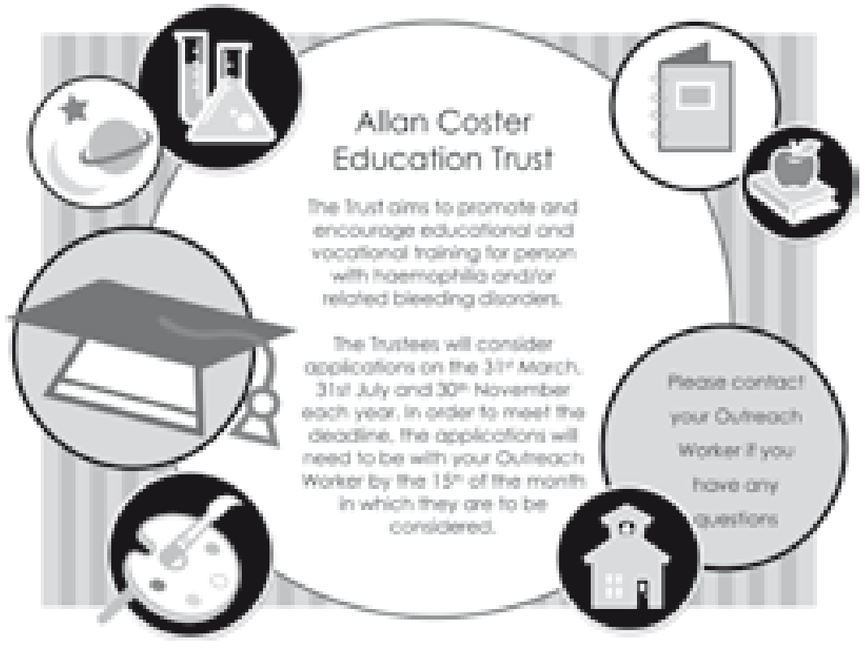





# Wellness Weekend a Winner!

***Building on the success of the Men's Weekends and the Couples' Weekends held in recent years, HFNZ held a National Adult Wellness Weekend on Friday 25st June to Sunday 27th June 2010 at the well located Copthorne Hotel Auckland HarbourCity on the Auckland waterfront.***


Wellness Weekend participants

Having a bleeding disorder impacts your health and wellbeing in a variety of ways and that can change over time. The aims of this weekend were to increase participants' knowledge on aspects of their bleeding disorder and learn ways of improving their wellbeing or hauora. All adults in New Zealand with a bleeding disorder were invited to attend. Partners were also welcome in recognition of the big part they play in health and wellbeing. In total, we had 25 people with bleeding disorders and 17 partners participate, along with all the Outreach Workers. Ages ranged from 22 to 70 years.

The concept of wellbeing encompasses the physical, mental and emotional, social, and spiritual dimensions of health. This theme threaded through the whole weekend and was introduced in the first session on Friday afternoon. Patience Stirling gave an overview of *Te Whare Tapa Wha*, a Māori philosophy towards health based on a wellness or holistic health model. In *Te Whare Tapa Wha* wellness is depicted as a four-sided house representing four basic beliefs of life as the walls: *Taha Hinengaro* (psychological), *Taha Wairua* (spiritual), *Taha Tinana* (physical) and *Taha Whanau* (family). If one wall falls down, the whole house can collapse. Haemophilia and other bleeding disorders impact not only on physical health, but on all aspects of health.

Over the weekend, participants had the chance to think about and discuss with others how their bleeding disorder has had an impact on them, their families and their life.

Saturday's programme focused on physical wellbeing and the complications that come with living with a bleeding disorder, including sessions on physiotherapy, pain management, and hepatitis C. Sunday's programme focused more on mental, social and emotional wellbeing, such as relationships, advocacy, and communication.

Some of the guest speakers included:

- Denis Jury, Chief Planning and Funding Officer of the Auckland District Health Board
- Dr Paul Ockelford, Haemotologist at Auckland Hospital
- Ian D'Young, Haemophilia Physiotherapy Practioner
- Prof. Ed Gane, Hepatologist and Hepatitis C specialist
- Kate McCallum, Pain Management Nurse Specialist
- Mary Brasser, Haemophilia Nurse Specialist
- Andrew McLean, Health & Disability Commission Advocate


Willy TeKira and John Wrathall


Sally and Bruce Marnoch


Rob and Jocelyn MacIntosh

*"Thoroughly enjoyed the chance to spend the weekend with a wide range of ages yet many shared experiences. Very informative and relevant speakers covering important topics in particular ageing with a bleeding disorder. Was also good to involve spouses and recognise the important support they provide. We appreciate the energy put into the weekend by sponsors and the HFNZ staff. Hope you are well - Blair Wightman."*

# Wellness Weekend a Winner!....

Dylan Shadbolt and Freya Kroupa

Mike and Cheryl Carnahan

Bruce Marnoch and Rob MacIntosh

Unfortunately, Brian O'Mahony was unable to speak during the weekend. The evenings were nevertheless enjoyable as the group was welcomed with a heartfelt Powhiri organised by Northern Māori members on Friday night and a ferry-ride over to dinner at the Esplanade Hotel in Devonport on Saturday night.

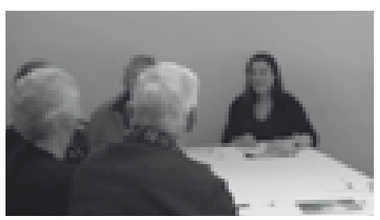
Overall, the Wellness Weekend was a winner. It was great chance catch up with old acquaintances and meet new people. The format and location meant that a number of people attended an HFNZ event for the first time. Thank you to all that attended and contributed to the success of the weekend. Of course, weekends like this would not be possible without our guest speakers, as well as the hard work and extensive planning of Outreach Manager Colleen McKay, Administrator Leanne Pearce, and the Outreach Team.

HFNZ are planning another educational workshop for adults in 2011. We want your feedback on the format this workshop should take. Keep a look out in the post for a survey we will be sending so that we can gather your thoughts and feedback.

Colleen McKay advising us all of the house rules

Te Whare Tapa Wha: model of wellbeing

> *It is always good to hear what developments have and have not been made (gene therapy?) in the fields of haemophilia and HCV treatment.*
>
> *Prof Ed Gane's talk was particularly encouraging for those like me who have tried once to clear the virus, without success. I have known these new treatments were coming, but the wait is often long and agonising.*
>
> *We should thank our lucky stars for Ian D'Young arriving in Auckland! His pro-active attitude to physiotherapy for haemophiliacs is refreshing and - I can personally attest - very effective and empowering.*
>
> *Thanks for organising the weekend. It went well and was a great success.*
>
> *Mike Mapperson.*



> *I attended the Wellness Weekend 25 to 27 June 2010 in Auckland. This was a great time to meet people with Haemophilia and their partners of the mature age group. There was time to talk to each other and swap experiences in our lives, how it impacted on the lives of us, our partners and children etc.*
>
> *The education sessions and speakers who spoke using short words that each of us could understand (not words 2 metres long) were very good and everyone could leave with a much better understanding of the treatments etc which affect our lives.*
>
> *It was great to just sit and talk to everyone learning, making new friends, going across Auckland Harbour on the ferry in the dark, seeing the lights of the city.*
>
> *Well done on the organisation of a very enjoyable and informative weekend.*
>
> *Rob McIntosh.*



# Introducing **Joy Barrett**, Midland Haemophilia Outreach Worker

Joy Barrett,
Midland's new Outreach Worker

Greetings,

I am Joy Barrett, the new Midland Outreach Worker. I am based in Hamilton where I have lived for the past 10 years, after 11 years in Tirau. After a really quick whirlwind landing in the position left by Sarah moving to Auckland, I am excited by all the wonderful people I have met in the Foundation so far; National Office Staff and the other Outreach Workers, speakers and attendees of the Wellness Weekend in Auckland, those who ventured out for Sarah's farewell in Tauranga and the hospital staff based here at Waikato Hospital.

I am recently separated after 28 years of marriage; have two adult daughters and two beautiful grandchildren all living in Hamilton. I am originally from Christchurch so having National Office in Christchurch has given me a chance to see my elderly mum too.

My background has been heavily focussed on community-based programmes through church programmes. Alongside that I have been working in the counselling field since 1995 in a variety of settings, so I have lots of contacts in the Waikato.

I am really passionate about encouraging those around me into not becoming a victim of whatever life throws at us. Life can sure throw some strange things our way. Sometimes it means getting extra help for a while. Acknowledging not only what is happening now is painful, but also the loss of the hopes and dreams that we hold so dear for the future too. I look forward to helping support those of you in Midland through those tough times and the good ones as well.

Joy B

# **Re-PEP** By the participants of Re-PEP

## "I learnt a lot about myself and how to be a better parent"

## Introduction

On the weekend of 7th / 8th August 2010, 15 Parents Empowering Parents (PEP) graduates reconvened in Christchurch to re-visit the PEP Programme. Participants from Auckland, Nelson, Christchurch, Hawkes Bay, Lower Hutt, Hamilton, and Blenheim travelled in the rain and cold to be together. Colleen McKay, Daryl Pollock and Linda Dockrill joined together to facilitate the structured Re-PEP Programme – which we believe to be a world first.

Diversity – all bleeding disorders, all walks of life, different structured families, parents with children of varying ages came together to benefit from this weekend.

***"I got to laugh and enjoy the company of others who understand"***

## About Re-PEP

Re-PEP was held 2 years after the first PEP Programme in New Zealand and 12 months after the second. Over the two days of the programme, the aims were to reflect and refresh ideas learned at PEP, as well as reconnect with other parents.

The weekend started with a Māori Welcome and Karakia by Rev Whare Kaa, who also has a grand-daughter with haemophilia. Bayer generously continued their sponsorship of PEP and thanks go to their representative Christie Murzello for attending on Saturday for the Official Māori welcome and staying on to join us for lunch.

The social event at night was fun and a good chance to talk informally.

It was great to realise we had actually implemented a lot of the ideas and tools that we had learnt without realising it, even though many of us had not re-visited our Manual. The groups were felt comfortable to share intimate information and everyone felt like they were in the same boat and we were able to offer mutual support.

***"I have a deeper connection with some special members of the Foundation from throughout New Zealand"***

Facilitators Colleen McKay, Daryl Pollock and Linda Dockrill

## How PEP has benefitted our parenting

PEP has helped us individually to realise the goals we set for ourselves. Those we had set for our families have been achieved sub-consciously, and the Re-PEP has helped us to realise this. Most of us feel that we have been guided to empower our children to be honest, independent and self-reliant people who are able to make sensible and well thought-out choices for their own safety, well-being and role in the family's wider community. We would recommend PEP to all parents with or without bleeding disorders.

***"I feel empowered, supported and no longer isolated"***

## Why you should attend the next PEP

- Life changing experience: a chance to assess your life and your parenting style.
- Contact / connect with other haemophilia parents / whanau.
- Empower yourself / relationship.
- It's not all about bleeding disorders.
- Chance to share experiences.
- It is hard work, but very rewarding.
- Free weekend away from kids.

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Originally developed in the USA, the PEP Programme was designed to educate parents and improve confidence in parenting skills. The programme is presented to parents by parents of children with bleeding disorders, in tandem with a social worker and nurse. This team approach combines the strengths of peer support with professional expertise. HFNZ is planning on running their third PEP Programme in 2011, so keep your eyes and ears open for registration details. For further information in the meantime visit **www.pepprogram.org** or speak to your Outreach Worker.

Carlin Goodwillie and Tania Kaa

Deborah and Gary Honnor

Kelly Vivian, Jolie De-Gaia and Jo Turner

Re-PEP mums proudly holding photos of their children



# In Remembrance

In the first half of 2010, HFNZ lost three long-standing members of the bleeding disorder community. HFNZ regret to announce the passing away of -

**John Hanssens** (originally of Northern but recently of Christchurch),
**Barry Mabey** (Feilding) and
**Ian Hammington** (Upper Hutt).

Our thoughts are with their family and friends and we hope to publish further details of their lives in a future issue.

# Waiata Competition

## HFNZ are running a competition for developing our own Waiata.

Waiata or songs and chants are an important part of Māori culture. There are many forms of waiata used for different purposes including oriori (lullabies), waiata tangi (laments), waiata aroha (songs of love), ngeri (a type of chant), manawawera (a form of challenge) and waiata poi (poi songs). When performing a waiata, it is important to choose a song appropriate for the occasion.

We would like to have a waiata that is uniquely represents HFNZ and our community. In the future, we can all sing our waiata at HFNZ gatherings or when representing the Foundation.

The waiata should try to include certain keywords such as haemophilia, foundation, New Zealand, Life, Love/Aroha/ Strength, and Whanau/Family.

The song can be original or based a well-known tune.

Entries must be received either by 15 October either to HFNZ National Office (PO Box 7647, Sydenham, Christchurch 8240) or by email to **info@haemophilia.org.nz.** The Roopu will choose the winner at their next meeting and we plan to publish the winning waiata in the December issue of Bloodline.

# Letters to the Editor

Bloodline features a Letters to the Editor section. This gives you the members, health care professionals and other interested members of the public a chance to share your opinions, ideas and experiences with the readers of Bloodline.

Please send correspondence to **info@haemophilia.org.nz** or by mail to Editor - Bloodline,
HFNZ, PO Box 7647,
Sydenham,
Christchurch 8240.

Bloodline is published in March, June, September and December of each year. Correspondence must be received at least one month prior to publication month to be included in current issue, otherwise letters may not appear until the following issue. Please keep letters relatively brief (250 words or less).

*Dear Editor,*

*When I last renewed my Mobility card I didn't have to get a doctors certificate as I have a permanent disability. Also for an additional $5 I was able to get a second card to use in other vehicles. I was always leaving my card in the other car or on the kitchen bench so I have found this very useful!*

*Regards,*
*Grant Hook*

# Big OE with Haemophilia (Part 2)

**By Michael Danusantoso**

Mike and his son visiting Oxford

Mike and his son in London

***In the first part of this article (from Mar 2008), I wrote that I was in my OE with my wife and young son. After a couple of years in the UK, we are finally back here in Wellington. We came back in October 2009 just before Labour weekend. Luckily for me, I managed to secure a job before I came back so I could start working on the Tuesday after – no rest for the wicked!***

After a few years, a former flatmate of my wife stayed over for a few days with us in the UK. We started talking about NZ and how different it was with the UK – that's when I started to realise how much I missed NZ. Sure, the UK has got plenty to give but there are things in NZ that just can't be replaced. I guess I didn't quite realise how lucky we are all living here in NZ until I moved to abroad.

In the over 10 years of my working live in Wellington, I have always kept my commuting time to under 30 minutes one way. During my last 6 months in the UK we lived in a house about 40 miles away from work! Initially I thought this was fine, people commute even longer distances, but after several months the commute started to take its toll. It took me a minimum of 40 minutes on the motorway (M26/M25) on a good day. Total commute time was approx 50 minutes one way even though I left at 7 am in the morning. At least once a week there was some delay on the motorway (be that an accident, some stupid rubberneckers, people changing lanes, wet condition, you name it) - the worst was one morning where it took 3 hours to get to work!

In my personal opinion, things are much more complicated in the UK because of bureaucracy. As mentioned in my previous article, if you're renting a house then you're responsible to pay council tax. We once just moved into a rental house and I couldn't remember whether I've paid my council tax or not. So I rang the council office, "Can you please tell me whether I'm behind or ahead with my council tax, I live in …etc etc". The answer was "It'll be posted to you". Regardless how much I tried to get the answer "behind" or "ahead", the response is the same – "It'll be posted to you". It's a good way to learn to be patient!

In some respects I found treatment for people with haemophilia in the UK cheaper. Like here, product is available through the public health care system. What's great, however, is that it's delivered to any address of your liking and they ring a week before to confirm. There's also a dental service too, free of charge. You get to see the specialist in a clinic once every few months for a check up.

However, there's a catch!

When I got back to NZ my haemophilia nurse informed me that I'm eligible for a dental service at the hospital…for a reduced fee (but I still have to pay). I actually had to go back a few times because they found cavities (i.e., more than one!) in my teeth. I wonder why the dentist in the UK didn't see them even in my last visit was only a few months earlier! Was I really saving on the dental service there? I don't think I'll ever know!

I must admit that UK has still got a lot to give. Castles abound, there are theme parks everywhere, museums, etc. My wife especially loves the shops in London. She says that clothing there is cheaper and more stylish. The internet is 2-3x faster than here and more reliable (just be aware that it took them 3 weeks to install!). Standard TV programming sucks though – I lost count how many times I watched the same episode of CSI over and over again. Groceries are cheaper (assuming you earn in £ as well), not to mention everyday goods (we were astounded to find the exact same outdoor table/chair set for sale at Briscoe's for $400 that cost £60 at Argos).

It has been a very eye opening experience for us. I would definitely recommend it for anyone who would like to get some experience overseas. If you're single, you'd probably like it even better (no kids to worry about). But, even if you have kids, I don't think it should stop you from doing your OE – we went there in 2007 when our son was less than a year old!

Best of luck! If there's anything you want to ask, feel free to email me at **mike.danu@gmail.com**.



# Tender Training with Brian O'Mahony

**By Belinda Burnett,**
**HFNZ CEO**

***Whilst the Wellness Weekend was happening downstairs, upstairs Brian O'Mahony was delivering a "Tender Training" course to a group of people from the National Council.***

Using the philosophy of "getting more bang for your buck" the Foundation held two events in the same venue, at the same time.

Over the weekend of Saturday 26th & Sunday 27th June 2010, the Tender Training discussed different aspects of 'Concepts in Factor Replacement Therapy'.

You may recognise Brian's name as he has held many important roles in the haemophilia world:

- World Federation of Haemophilia President 1996 – 2004
- Irish Man of the Year 2003 (for services to people with Haemophilia and Hepatitis C)
- Chief Executive Officer of Irish Haemophilia Society 2005 – present
- Creator of the Global Alliance Programme (providing safe product to developing countries)
- Sufferer of Severe Haemophilia B

Brian also played a crucial role in advancing HFNZ's progress in achieving the Treatment and Welfare package for people infected with hepatitis C through blood products.

The Tender Training programme consisted of:

1) Basic concepts in replacement therapy looking at the history through to current recombinant products
2) Safety - the history and current and future threats
3) Economics – per capita use and cost through to evidence based medicine
4) National Tender Systems, from types to selection criteria and the advantages and disadvantages of each
5) World Federation of Haemophilia Regulatory Guide
6) Conducting a Mock Tender for purchase of recombinant products within a country with higher defined GDP per capita and per capita use
7) Discussion and feedback

Attending the course was Deon York (HFNZ President), Catriona Gordon (HFNZ Vice President), Richard Scott (Northern Delegate), Mark Uren (Southern Delegate), James Poff (Southern Treasurer), as well as Belinda Burnett and Chantal Lauzon (HFNZ staff).

This course is aimed at helping members of haemophilia societies in developed countries play an informed and involved role in their tendering process, and delivers a haemophilia specific focus. New Zealand is the forth country Brian has delivered this course to.

In 2011, the third PHARMAC tender will be held to secure the supply of recombinant factor VIII products in New Zealand. PHARMAC first contracted for the national supply of recombinant factor VIII in 2005 – prior to this District Health Boards contracted for their own purchases. National contracts are currently in place with three suppliers of recombinant factor VIII (Bayer, Baxter and Pfizer). Next year, PHARMAC will again be looking to secure the supply of recombinant factor VIII for a period of 2-3 years to ensure those in the haemophilia community are able to access appropriate, timely treatment. HFNZ also want to make certain that those in the haemophilia community are able to access not only appropriate, timely treatment but also the safest and best available. Participating in the training course is one way to ensure we have the necessary knowledge and skills to be part of the decision making process.

# Southern Girls Day Out

**By Linda Dockrill,**
**Southern Outreach Worker**

***In mid-July a "Southern Girls Day Out" was held in Christchurch. This was a fun and educational day for young women aged 10-15 years who have bleeding disorders or who carry the gene for a bleeding disorder. Young women from as far North as Nelson and South as Invercargill came together to meet and get to know each other.***

We started the day with hot chocolate and muffins while the mums and grandmothers were whisked away for coffee and cake at Riccarton Mall. We were joined by Alison Inder, Clinical Nurse Specialist at Christchurch Hospital, who talked to us about girls' bleeding issues using each young woman's individual case history as her starting point for information. The older young women who had come in a support role were also able to learn heaps too.

After a delicious lunch the girls and their mothers/grandmothers settled in for some creative activities and produced some lovely bead jewellery and attractive paua photo frames.

Later in the afternoon we practiced our salsa and dance moves at a Zumba class. We were joined by some other young women from the local community and had a lot of fun trying to move our bodies in time with the music. Some new moves have been created in the dance world as a result!

# Pharmac approves subsidy of meloxicam for pain relief in haemophilic arthropathy

The following is for information purposes only and not intended to take the place of medical advice. Treatment decisions for all health conditions related to your haemophilia should be made together with your haematologist or specialist.

***From 1 September 2010, meloxicam will be funded subject to Special Authority criteria for patients with moderate to severe haemophilia and pain and inflammation associated with haemophilia arthropathy (joint disease) where alternative funded treatment options have failed or are contraindicated.***

Arrow-Meloxicam 7.5 mg tablets will be listed at a price and subsidy of $11.50 per 30 tablets (excluding GST). The usual dosage is one 7.5 mg tablet per day. The funding of meloxicam will be subject to the following Special Authority criteria:

1. The patient has moderate to severe haemophilia with less than or equal to 5% of normal circulating functional clotting factor; and
2. The patient has haemophilic arthropathy; and
3. Pain and inflammation associated with haemophilic arthropathy is inadequately controlled by alternative funded treatment options, or alternative funded treatment options are contraindicated.

## What Meloxicam is used for

Arrow-Meloxicam is used treat symptoms of osteoarthritis and rheumatoid arthritis. Both diseases mainly affect the joints causing pain and swelling. Meloxicam can relieve symptoms such as pain and inflammation (swelling, redness and soreness) but will not cure the underlying causes.

Meloxicam is a non-steroidal anti-inflammatory drug (NSAID) which has shown anti-inflammatory, analgesic (pain relief) and anti-fever properties. Meloxicam is one of what are known as COX-2 inhibitors. NSAIDS which are COX-1 inhibitors (aspirin, ibuprofen) are generally not recommended for people with bleeding disorders, however, it is believed that COX-2 inhibition provides the therapeutic effects of NSAIDs without the gastric and renal side effects, or the inhibition of blood clotting associated with COX-1s. Meloxicam has been demonstrated to have no effect on either platelet aggregation or bleeding time at recommended doses. It is currently the only COX-2 inhibitor with Pharmac funding for haemophilic arthropathy.

As with any medication there are possible side-effects and contra-indications. Speak to your doctor about allergies to other medicines and other medical conditions, especially high blood pressure, other heart problems or a history of gastrointestinal disease (ulcers, etc). Meloxicam is also contraindicated in children and people with severely reduced liver function.

# Regional Branch Reports

## Northern

By Lynley Scott

The past few months have been quiet for the Northern Branch in terms of social activities but there has been plenty of planning going on within the committee. We are excited to welcome our new Outreach Worker, Sarah Preston, and look forward to working together with her to reach out to the Northern folk. Sarah definitely comes with a passion and enthusiasm for haemophilia so no doubt we will all benefit from that. By the time this goes to press we will have had a dinner together to welcome her, hope you all were able to make it.

The national Wellness Weekend was held in Auckland recently and sounds like a great time was had by all. Lots of time to connect, be educated and have fun.

Richard and Lynley Scott attended WFH Congress in July in Argentina which was by all accounts an amazing time, see the reports in this issue of Bloodline.

Over the next few months we will have a few social activities – Butterfly Creek for the families in early Sept and the annual Movie Night for Global Feast in mid Sept. Again we hope that you can join us for those events. We'd love to hear from you regarding other events that you might like to see run. And of course the annual Christmas Party in December. Keep an eye for invitations to these events and if for some reason the invitation doesn't arrive please ask Sarah for more information.

We would love to next year provide opportunities for the Far North members to meet together, so if you are one of these families, watch this space or let us know some suggestions.

We look forward to touching base with all our Northern Members at social events and hope that you are drying out and warming up from the winter – roll on Spring, I say!



# Regional Branch Reports...

## Midland

By Catriona Gordon

Midland has been busy through the winter, firstly with a farewell café evening in Tauranga for Sarah Preston in June. We were all sad to see Sarah go, as she has been a wonderful Outreach Worker for our region, but we are very pleased that her skills and enthusiasm are being retained by the Foundation as she goes on to join Northern.

At the café evening we were also able to meet our new Outreach Worker, Joy Barrett. We are looking forward to meeting her individually, but some of us have already had the chance to see her again at various branch events.

At the end of July some of our members went to the Wellness Weekend in Auckland, which was a great educational event. At the same time some members of the council, including myself from Midland, had a training session with Brian O'Mahoney on tendering for blood products, which was very informative and a valuable opportunity to learn from one of the great leaders of the Haemophilia global community.

At the end of July Midland held a family day at Tauranga Baywave. In the past some members may recall we have been blighted by poor weather at our events, so this time we wisely played it safe by going to an indoors venue, where no trees were able to break the hydroslide in half, and the wave pool was pleasingly empty of leaves and branches. Everyone had a great time, and an enormous lunch, which might not necessarily have got the Heart Tick. At least, after sufficient time to allow for digestion had passed, most of the younger members were back in the pools and racing down the hydroslide, burning those calories off.

We have recently held a meeting to plan another Café Evening in Hamilton which will be held on 11 September and will incorporate the Global Feast fundraising effort. Planning for our Christmas Family Day in Rotorua on 7 November is also well underway, and we are starting to organise our Midland Camp to be held on the weekend of 4 March 2011 at Totara Springs in Matamata. We look forward to seeing as many members as possible at some if not all of these events.

## Central

By Stephanie Coulman

We are also very proud of Deon York's (we're claiming him as he is a central region member!) appointment to the World Federation Executive. Out of 112 national member organisations and only 12 positions, (6 medical and 6 lay) he is one of the lay members. It's an impressive achievement.

News from BJ Ramsay, Wellington Hospital's Haemophilia Nurse Specialist – a new initiative, although one BJ says we should have been doing already, is holding specific clinics once a year to review our ladies who are carriers of haemophilia. "It's particularly useful for women with low/borderline factor levels as these ladies have mild haemophilia," says BJ. Call BJ if you would like to be booked in to these clinics.

With haemophilia and ageing becoming a topic gaining more focus, once a month Wellington Hospital is running specific haemophilia/pain clinics. It is held in Wellington with Dr Paul Hardy, an experienced Consultant pain specialist. The clinics aim to view pain and its management as part of the comprehensive care team model rather than just a service when pain has gotten out of control. BJ says this service is available to any patient, regardless of age, who feels they would benefit. Again contact BJ if you would like to access this service.

The idea of missing out on a week away without mum and dad during the school holidays was enough push for 9-year old Liam Habershon to master self-treating. Without his parents to do his 3x weekly treatments he wouldn't have been able to go away. So congratulations on this milestone Liam! Who needs parents anyway!

Wellington Men's Evening with Brian O'Mahony in June

## Southern

By Theresa Stevens

Hello everyone,

Isn't it grand that the weather is just starting to improve – it makes be believe Summer is just around the corner.

This past weekend we woke up early to attend the Farmers Market and instead we were greeted with a deliberately lit fire in one of our vehicles... fire brigade and police with dogs started our morning and we still haven't made it to the market! Our car was one of two deliberately set on fire in Dunedin within a 24 hour period that weekend. It has been a particularly traumatizing time for our family, not to mention all the other folk affected by the many arsons around the city over the past month.

In addition to the Girls Day Out held at the end of July (see page 7), a get-together for families in the Christchurch region with children with bleeding disorders under 10 years was held at Lollipops Playland on Sunday, 4th July. This was attended by 6 families, including 10 children.

Planning for our regional family camp in January is well underway. The camp has a change of location this time as we head out to Hamner Springs. The Southern Branch Camp will be held 21-24 January, 2011 at Hanmer Springs Forest Camp. Forest Camp is located just 3kms from Hanmer Springs village and all the regional activities. Remember to book these days off work and book your place at the camp. As usual bookings will be on a first in first served base but we hope to accommodate everyone who wishes to attend. Camp isn't only for kids, all members who live in the South Island are welcome. There will be further information posted directly to Southern families.

Jayde Peat continues to succeed in his touch rugby – well done Jayde.

Sarah Morrison has recently returned from Australia where she competed in gymnastics – well done to you Sarah!

Michael Stevens deserves a mention here too (despite being my own son!) Well done Michael on achieving your Limited Electrical Registration exam.

Until next time I hope you all have a wonderful spring.

# News in Brief

## Cardiovascular Care Found to Be Lacking for Patients with Haemophilia

Patients with haemophilia and cardiovascular risk factors may not be treated aggressively enough out of fear of complicating their bleeding problem and many patients appear to be not sufficiently assessed for cardiovascular risks at all, according to experts in the field.

After a review of data on 18 patients with haemophilia and cardiovascular risk factors at 11 haemophilia centres, Antonia Coppola, MD, Regional Reference Centre for Coagulation Disorders, Naples, Italy, found that more than 80% of the patients had two or more established cardiovascular risk factors at the time of the review.

Dr. Coppola said that the treatment was "not adequate in most cases, because of the fear of bleeding complications. Nine of the patients were not being treated with antiplatelet agents, such as aspirin or clopidogrel. Of those who were treated with one or both of those agents, seven suffered bleeding complications, with three considered severe.

In the two cases with no complications, prophylaxis was given. In 13 cases, percutaneous coronary intervention was performed and was found to be feasible. All were accompanied by sufficient factor replacement.

Dr Coppola suggested that, "secondary prevention with antiplatelet agents should be given, in association with prophylactic regimes tailored on the basis of the clinical course."

In the ADVANCE study group review, data from 17 centres across Europe found that only three of the 17 centres surveyed assess cardiovascular risk using an established scoring system, such as SCORE or Framingham, and only a third of the centres assess risk at all. The review found that the centres seemed comfortable prescribing blood pressure and cholesterol-lowering medications, but not anti-platelet treatment.

Gerry Dolan, MD, Nottingham Hemophilia Comprehensive Care Center, Nottingham, United Kingdom, said the review shows the need for more education and collaboration.

"Few centres have access to cardiac expertise and services," he said, "and referral practices varied widely."

*SOURCE: Collins, TR on www.docguide.com at the Hemophilia 2010 World Congress*

## Acupuncture Eases Pain in Haemophilia Patients

Sticking needles into patients with bleeding disorder may seem counterintuitive, but researchers at the WFH Congress said that acupuncture appears to alleviate some joint pain commonly experienced by people living with haemophilia.

In a pilot study, six of nine patients achieved substantial pain relief, including one patient whose visual analog pain scale score dropped from 10 to 5, said Angela Lambing, MSN, nurse practitioner coordinator at Henry Ford Health System, Detroit. The trial was conducted in Detroit and in Karnataka, India, as part of the WFH twinning program that connects treatment centers around the world.

In the quality-of-life survey using the Short Form (SF-36) questionnaire, patients reported improvement in physical functioning, emotional problems, mental health, pain relief and positive changes in health.

"Importantly, we saw no bleeding in any person. We saw no bleeding after any treatment session. And we didn't see any bleeding if the patient was taking blood factor to prevent bleeding episodes or was not on those treatments," said Lambing.

It should be noted however the number of patients is really too small to do any meaningful statistical analyses, but Lambing considered it a successful trial.

Lambing said she really didn't expect any bleeding issues with the acupuncture needles because the instruments used are 36-gauge needles -- about the width of a human hair, and the needles do not penetrate all the way through the skin.

Adults diagnosed with haemophilia were eligible for the study if they reported chronic pain and were diagnosed with severe joint damage. They were treated with acupuncture at 20 sites, including specific points in the knee, ankle, lower back, and elbow for patients experiencing pain in those areas.

The acupuncture treatment program involved two sessions a week for four weeks, followed by weekly sessions for six weeks. Lambing said the long course of treatment probably reduced the enrollment in the trial.

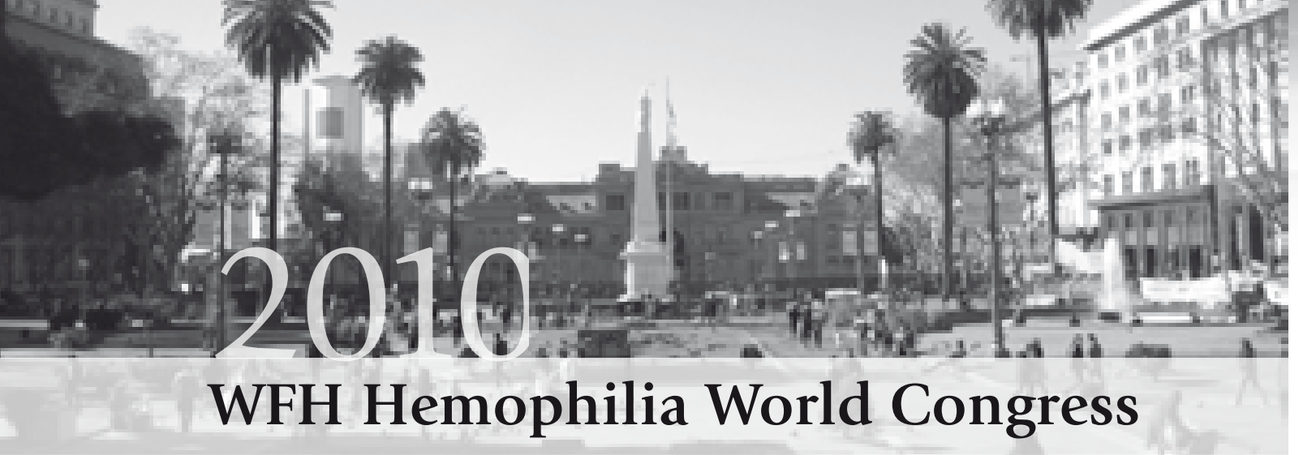
"Obviously we need larger randomized studies," Lambing said. "But as an alternative therapy, acupuncture may provide some benefit to chronic pain patients with hemophilia in a multimodal approach."

*Source: www.medpagetoday.com/MeetingCoverage/WFH/21162*





# 2010 WFH Hemophilia World Congress



# 2010 WFH Hemophilia World Congress

**The XXIX Hemophilia World Congress was held in the vibrant city of Buenos Aires, the capital of Argentina. Known as the "Paris of South America", it is the home of the tango and a place of gourmet meals (the beef and wine are world famous) and a lively, cosmopolitan spirit (read lots of people and lots of traffic!). The 2010 World Federation of Hemophilia (WFH) Congress took place from 10-14 July, and although this turned out to be the coldest week in a decade it did not dampen the atmosphere.**

With an attendance of over 4,300 participants from 106 countries, this was a record-breaking Congress. Buenos Aires is known as a meeting place of people and cultures and so was an ideal spot for the Congress, where those in the global bleeding disorders community come to share their knowledge, research, advice and experience. The WFH's mission is to improve and sustain care for all people with bleeding disorders, women and men, young and old, and those in developing and developed countries. The Congress highlighted the work that is being done around the world in the education and psychosocial support of patients and families, better organisation of care, training of multidisciplinary health teams and innovations ahead in clotting factor therapy.

In his opening speech, WFH President Mark Skinners noted the remarkable progress and success in diagnosis and care that has been achieved in many areas to date. It is easy to lost sight of the huge success the WFH have already achieved when there is so much still ahead. When the WFH Strategic Planning Committee developed the concept of "Treatment for All" in 2006 it seemed as if this was an elusive dream. In four short years much progress has been made in making this a reality for many people in developing countries. Skinner maintained, however, that much work still lies ahead, particularly for women with bleeding disorders, patients and families in sub-Saharan African and with youth.


A record number of HFNZ volunteers and staff were able to attend thanks to generous sponsorship from WFH, our Sustaining Patrons (Baxter, Bayer, CSL, NovoNordisk and Pfizer) and a donation from Roche Pharmaceuticals. Although the Congress is a fantastic place to just absorb information, us Kiwis weren't just spectators. HFNZ President Deon York, CEO Belinda Burnett and past-President Mike Carnahan were all invited speakers. New Zealand health care workers were also invited speakers, such as Alison Inder, haemophilia nurse coordinator at Christchurch Hospital and Clare McClintock, a haematologist from Auckland. Three posters from HFNZ members and staff were also presented:

- Lynley & Richard Scott: Salvage therapy with Rituximab for inhibitors: the parent experience
- Lorraine Porter-Bishop: 1 + 1 = 3: A family's experience of vWD Type 3
- Chantal Lauzon: A review of services and the needs of the bleeding disorder community in New Zealand

Although a small country, our enthusiasm shone through with New Zealand taking the prize for the most donations to WFH per capita of the home country made during the Congress. With every donation and new WFH membership you got to put a pin in the huge world map and New Zealand was proudly decorated.

One of the key reasons for attending the Congress is to bring back as much information as possible to New Zealand. Reporting back on the over 70 sessions was not possible, but in the following pages you will find reports on all the multidisciplinary sessions and many of the medical sessions as well. All the delegates hope that you will find them informative. If you would like more information on a particular subject please contact your Outreach Worker or Chantal Lauzon, National Information Coordinator at chantal@haemophilia.org.nz.





# Capacity Building & Care Delivery

## President's Address: Building our global family - achieving Treatment for All

*By Lynne Campbell*

To improve and sustain care beyond haemophilia to support all those with bleeding disorders – men and women, regardless of where they live in the world, was the very clear message from the President of the World Federation, Mark Skinner.

Our global family embraces the many faces of bleeding disorders. The President's address acknowledged the success in diagnosis and care in many areas, but focussed particularly on building our global family by way of recognising and incorporating treatment for women with bleeding disorders, children and youth. He equally promoted the need for more work to be done to deliver diagnosis, training and capacity in the developing countries of sub-Saharan Africa in order to achieve Treatment for All.

Skinner emphasised that The World Federation's mission to improve and sustain care "goes beyond haemophilia to incorporate advocacy and support for all people with inherited bleeding disorders".

Women with inherited bleeding disorders such as rare factor deficiencies, inherited platelet disorder, von Willebrand's Disorder (vWD) and carriers of haemophilia often have no idea their symptoms are abnormal even though they impact significantly on their quality of life.

Skinner's considerable focus on women with inherited bleeding disorders acknowledged:

- The challenges for women experiencing a reduced quality of life and the impacts on them professionally and socially in day-to-day life. Often, in the absence of other treatment options, women underwent early hysterectomy.
- The gender split for bleeding disorders outside of haemophilia is equal. For example for vWD, the most common bleeding disorder in the world, data collected since 1999 indicates 40% of people with vWD are men and 60% are women. The reason for this is most probably because woman present with complications related to being female, such as menorrhagia. It is estimated the incident of vWD is as high as 1.3% of the world's population.
- Carrier women of haemophilia are a significant and often neglected group of women. 20% of carriers have blood clotting levels below 30% (mild haemophilia) and there are up to two million carriers in the world. For every 1500 people with haemophilia it is estimated there are 4,000 relatives with carrier status and thus in potential need for support.

Carrier women tend to go undiagnosed or improperly managed. Lack of awareness by caregivers often delays proper diagnosis and treatment when women do seek help. Post partum haemorrhage is the main cause of maternal death and long term disability in carrier women. (There is no evidence to support that this is the case for women with vWD).

Skinner noted that women typically first present with clinical symptoms at a community based medical facility, therefore there needs to be increased awareness within the broader medical community in order for women to receive appropriate education and guidance.

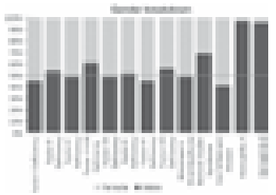


**Figure 1.** Proportion of male and female patients for all bleeding disorders. The white line indicates 50%. These data are from the 72 countries that provided gender breakdowns to the World Federation of Hemophilia Global Survey 2008.

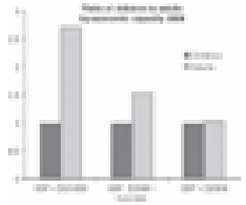


**Figure 2.** Relationship of economic capacity to number of adults with haemophilia. Comparison of the ratios of the number of adults with haemophilia to the number of children (aged <13 years) with haemophilia in 76 countries grouped according to per capita gross domestic product (GDP) in US dollars. The data were reported by national member organizations in the World Federation of Hemophilia Global Survey, 2008.

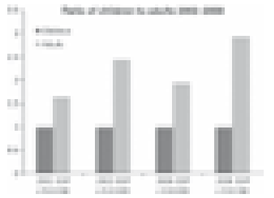


**Figure 3.** Change over time of relationship of economic capacity to number of adults with haemophilia. Comparison of the ratios of the number of adults with haemophilia to the number of children (aged <13 years) with haemophilia in 39 countries grouped according to per capita gross domestic product (GDP) in US dollars. The data were reported by national member organizations in the World Federation of Hemophilia Global Survey, 2002 and 2008.

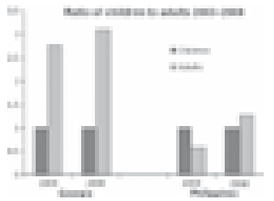


**Figure 4.** Comparison of the ratios of the number of adults with haemophilia to the number of children (aged <13 years) within Georgia and the Philippines before entry into the World Federation of Hemophilia (WFH) Global Alliance for Progress project and year-end 2008. The data were reported by the national member organizations in the WFH Global Survey, 2003 and 2008.

Medical personnel at all levels will be central to WFH outreach efforts and integral to the multidisciplinary model of care.

Addressing this imbalance has implications for haemophilia treatment centres, global outreach and genetic counselling. From WFH's perspective, the next crucial steps include the global development of outreach and registry programmes to rapidly identify women with inherited bleeding disorders and "educate and guide them to the appropriate clinical care setting".

WFH is gradually yet discernibly implementing targeted national development programmes regionally into Africa. The expansion of training capacity of WFH's sustainable national care programmes for patients with bleeding disorders into Africa builds on achievements in South Africa, Senegal and Kenya. Currently 15 of 53 African countries are national member organisations (NMO's) making sub-Saharan Africa the most under represented geographic area within the WFH.

Through Global Alliance for Progress (the GAP programme) WFH regional development has focussed on providing regionally based medical, educational and psychosocial programmes via training local personnel. Children and youth with bleeding disorders in low income areas are the primary target for closing the gap between the number born with haemophilia and those that survive to adulthood.

WFH has identified that children with bleeding disorders in low income countries are at risk of dying young and not reaching adulthood. Data gleaned by WFH confirms that as economic capacity of a country decreases so does the ratio of adults to children.

Through encouraging countries to network with each other and organize a variety of culturally appropriate programmes on a regional level, the work of the WFH is maximised. Five of the 11 sub-Saharan countries now have patient registries. WHF plans to establish medical twinning partnerships in all the member countries within the region. Twinning is the vehicle for core medical expertise and care and ultimately for a network of treatment centres to be established. Once a sound infrastructure is in place the WFH the promise of Treatment for All can be realised.

## Twinning: overcoming the barriers

*By Sarah Preston*

Twinning is a two-way collaboration between countries. One country with experience, leadership and resources, and one that needs the former. The aim is to enhance the global movement of haemophilia worldwide, to build capacity of the less able country, to coach and assist them and to share best practice.

Once countries are 'matched', there is an assessment visit to look at the less able country's needs. Goals are set and strategic plans are made between the two countries on how to best achieve them. Twinning can last up to four years and WFH help fund the project. Visits are made to the country and activities take place such as training medical staff, patient education, conferences, workshops, camps, treatment protocols, lab protocols, consultations and resource donating.

The aims are to assist better and more comprehensive haemophilia centres/foundations and better practices. There can be issues such as communication, language and cultural barriers, as well as unrealistic expectations, time and resource availability.

Twinning requires a team approach and a passion to help others succeed.

## Personal and Social Development of People with Bleeding Disorders

*By Lynley Scott*

First, David Silva (Spain) gave a presentation on the **Role of the NMO Defending People's Rights**. David, who has mild haemophilia, discussed the importance of the involvement of the National Member Organisation (NMO) in advocating for the person with haemophilia. He outlined that all countries has legislation around peoples/patients rights and responsibilities (in NZ, this would be the Health and Disability Act) and additional ones that the person with haemophilia has like the responsibility of high cost treatment, benefits of effective treatment, etc.

After this introduction, David went on to discuss the roles that the NMOs should play. These included:

- Educational/Training Role – especially important in the Haemophilia Field where it is so small and specialised.
- Information and Advising Role – a patient that is well informed makes for a responsible patient.
- Psychosocial Advocacy – this is not always supplied by the Health System.
- Promotion/Defense of Rights – an essential role both in individuals and for the collective community.

David listed three examples of the ways in which NMOs have worked in this role:

1. IRELAND – Life and Mortgage Insurance (and Travel Insurance) for HCV/HIV patients. This took 7 years and had a successful outcome.
2. MEXICO – Comprehensive care for Paediatric patient in Social Services. This has been ongoing since 2009 and individual patients have pressed charges against Health Authorities.
3. SPAIN – Recombinant products as first call for treatment. This took 4 years to achieve a successful outcome.

Next Antonio F Gomez (Argentina) spoke about **Prospects and Job Opportunities of Persons with a Bleeding Disorders**. Antonio began the session outlining the limitations that may face persons with bleeding disorders (PWBD) and that limitations were not necessarily to physical aspects but could include social and emotional areas also. He also felt that NMOs should provide opportunities for social networking, career guidance and advice.

He outlined how work and employment prospects are important in the social development of PWBD as this de-emphasises disability and limitations and makes the bleeding disorder not the sole focus of life.

In Argentina, 4% of companies' employees should have a disability. The NMO has developed an area and time to discuss job/career options with patients which includes skill and knowledge assessment. The NMO generates opportunities to develop CVs for jobs.

Conclusions from the session were that we need to rethink the concept of disability and approach it holistically; NMOs should be prepared to help, guide, and assist PWBD in the area of jobs/careers, and the NMOs should ask for assistance from Government, Health and Employment places.

Finally, Susan Cutter, a Social Worker from the USA discussed **Psychosocial Care and Education for the Patient with Bleeding Disorder**. Susan began the session by outlining the psychosocial considerations that needed to be considered for the patient with a bleeding disorder. The potential impact of the diagnosis on the person can be affected either positively or negatively by the severity of the disorder, their symptoms, their self esteem, their support system and their socio-economic situation. Cultural, religious, familial issues affect the person's perception of the disease, symptoms and treatment which can impact their decisions around care and treatment, disclosure, family planning, and activities and sport. Susan gave examples of how culture can impact decisions of disclosure leading to inadequate care and treatment – cultures seeing it as a 'white man's disease' or the importance in some cultures placed on sports and masculine activities. In some situations, seeking treatment may go against the familial, cultural or religious norms leading to complete isolation. The emotional adjustment of the person with a bleeding disorder can be influenced by their previous coping strategies (either positive or negative), their family, or other pressures. Maintaining a balance within the family is difficult and the bleeding disorder should not be the primary focus of the family. She also discussed how coping can erode over time and while someone could be perceived as previously coping, something seemingly insignificant can tip the scales to cause someone to not cope. An area often not explored is the limitations arthropathy can cause including difficulty with socialisation (at school or work), body and self-esteem issues, a change in the persons role, a sense of normality, relationships and pain issues. While the arthropathy may be a physical symptom, it can cause significant psychosocial issues. There has been much research around Quality of Life (QoL) issues of the person with Haemophilia and Susan discussed a number of these – majority of the QoL issues were in the physical domain, although a number of children and adolescents reported it to be higher in the social domain. QoL scores consistently tended to be higher in those children or adolescents who were on prophylaxis. One study she discussed was a Canadian study of 26 females with von Willebrands disorder (Barr et al, 2003) who had lower QoL scores in the areas of emotion, cognition and pain and that the women has QoL scores similar to men with haemophilia and HIV. The author speculated that this could be due to gynaecological issues. With regards to educational attainment, the person with a bleeding disorder had a similar outcome to the general population, however, those who bled more often had a lower attainment in reading and math. Susan summed these all up by saying that "a patient's ability to successfully cope with and incorporate challenges they face with their bleeding disorder will impact the choices they make, throughout their life cycle, including those related to their: relationships, family planning, physical activities, adherence to medical care and treatment, and careers and educational attainment.

Susan expressed that strategies around psychosocial issues of the person with the bleeding disorders should include strengthening the internal locus of control and positive coping strategies. Some of this can be done by encouraging open communication with all involved in their care (families, medical staff, etc), positive peer relationships, participation in activities and sports as able, and attention to the individual and collective needs of the family, encouraging people to focus on what they can do, reminding people that their bleeding disorder is just a part of their whole person, assist in transitioning through life cycles, providing age appropriate information and guidance, fostering patient decision making and advocacy skills, and helping people to understand the need to maintain or restore balance within their family system.

## Importance of psychosocial support in the treatment of people with bleeding disorders and their families

*By Grant Hook*

When treating people with haemophilia (PWH) it is important to provide complete psychosocial support not just medical support for the bleeding disorder. By understanding the patient's background, support structure, family, social and work situations health professionals can tailor solutions and understand issues more fully.

Outside influences affect the success of treatment and therefore the patient's overall well-being. Factors such as levels of social engagement, family support/stresses, housing and work issues all impact on a person's well-being and ability to maintain treatment regimes. If the health professional can get a complete picture of the patient's situation then these factors can be taken into account with treatment. It is also up to the patient to help by giving full information to assist in this. We must remember that few health professionals get sufficient funding to assess all of these factors and therefore they often get missed. With the introduction of more haemophilia nurses I believe we have a better opportunity for our health professionals to gain a complete picture and to be able to provide a better service to PWH.

## Integrating Outreach to Women with vWD and Rare Bleeding Disorders into NMO Strategies

*By Lynne Campbell*

This interactive session was an informal mix of speakers from a range of different countries describing what was being done in their own country for women with inherited rare bleeding disorders (RBDs).

- Chair: Clare Cecchini, Canada
- Canadian Experience - Clare Cecchini
- Lebanese Experience - Claudia Djamas-Khayat
- Georgian Experience - Marina Mdivnishvilia
- Venezuelan Experience - Arlette Ruiz-Saez

The common themes were:

- Outreach awareness campaigns involving the media, health authorities and lobbying government.
- The use of questionnaires and training female volunteers to mentor other women with RBDs to encourage registration and participation in countries where women were reluctant to disclose their RBD status and were reluctant to be tested.
- Gynaecological and obstetric management.
- Targeted (easily understood) information distribution for life stage issues for those living with rare bleeding disorders.
- National, culturally appropriate women's workshops and educational camps for women to share their experiences.
- Establishing women's multidisciplinary programmes.
- State of the art conferences on specific bleeding disorders.

It was interesting to note that in many regions of the world, both developed and in developing countries, women tended not to act upon their symptoms and seek medical help. In developing countries treatment reagents are expensive and only in extreme situations would the women be tested. Women were afraid to have their medical circumstances known because of the perception they would be perceived as unsuitable for marriage - or be labelled with some other stigma.

In many of the poorer developing countries represented (such as Georgia, Lebanon and Venezuela) haemophilia organisations and societies had previously not been considered a priority and had only been developed in relatively recent history (since the year 2000). In regions of political instability such as Georgia the importance of the political situation was dominant and outreaching patients to identify and assess need was limited.

In developed countries such as Canada, the number of women prepared to have their symptoms addressed and are formally registered with RBDs has gone up dramatically. Educational publications, interactive videos and the use of the internet and subject specific online resources such as information for adult carriers, teen carriers, vWD and other RBDs have all enhanced awareness, access to information and validation of medical symptoms.

Further information:

**www.wfh.org/2/docs/Publications/Hemo_Org_Resources/Patient-Outreach-Guide.pdf**

See Section 3 Case studies on patient outreach in each of these regions.

## Patient Outreach: Catch Them Early

*By Sarah Preston*

This session talked about the importance of diagnosis, inclusion of all bleeding disorders and ways to raise awareness and the profile of all bleeding disorders.

It focused on the Canadian experience, the Russian experience, the Mexican experience and the Tunisian experience. Although they are very different countries, they highlighted some similar and interesting points regarding 'catching patients early'.

There is under-diagnosis especially in woman with vWD, rare bleeding disorders and symptomatic carrers. Carriers often push aside their issues and instead keep the focus on their sons with haemophilia. Women with issues particularly relating to heavy bleeding during periods do not want to talk to their clinician about it. People with rare bleeding disorders can feel complete isolation.

To overcome these issues we must: keep a full and up-to-date registry of people with bleeding disorders; publish articles and use the TV and radio to raise awareness and encourage people to get tested; hold conferences and workshops to address the issues; and give these 'forgotten' people space to share stories and experiences.

## Development Steps to Establishing a National Hemophilia Care Program

*By Catriona Gordon*

After the first shock of receiving a diagnosis, children with bleeding disorders need reassurance and supportive care, and panelists in this session said doctors, parents, psychologists, and WFH national member organizations (NMOs) can all do their part to help.

"Why me?", "Why my child?", and "How will we cope?"are the first questions that come to mind for a parent who learns that their child has a bleeding disorder, said Cesar Garrido, vice president of the Venezuelan NMO.

The difference today is that parents can rely on an established set of strategies and support networks to protect their children and improve their quality of life. The most immediate coping strategies are to understand the disease, learn about the available treatments, and teach children to participate in their own care.

"By participating actively in local NMOs, parents can get the information they need…and learn to avoid feelings of denial and overprotective behaviours."

Gordana Stevanovic from Serbia said the best way for a parent to help their child cope with a bleeding disorder is to be honest and explain the condition, as early and as fully as possible. The child will have questions, and those questions need and deserve honest answers. Giving the child the whole story will help him or her build self-esteem and avoid any sense of guilt or shame about their condition.

By participating actively in local NMOs, parents can get the information they need, meet other families that share the same experience, connect with experienced healthcare providers, and learn to avoid feelings of denial and overprotective behaviours that can be counterproductive for the child.

Christine Keilback of the Canadian Hemophilia Society said family camps and parent-child activities are a great way to build close bonds and shared experiences in families living with haemophilia.

## Overcoming obstacles to comprehensive care in developing countries

*By Sarah Preston*

One topic covered was that of the role of new technologies of genetic diagnosis. Genetic testing helps us understand the biology of the disorder. Different types of testing were discussed. The main problem for developing countries is the lack of methods which are cost-effective and reliable. Alternative appropriate methods are being studied in India to ensure all countries can complete genetic testing and have full and accurate results and information.

Another important topic discussed was that of strategies of patient identification. The Philippines was used as a case study. The main theme was the importance of having a full, comprehensive and regularly updated registry. This is crucial to ensure we can track and support patients, and also as a tool to lobby governments for help or financial assistance (which is strengthened by numbers of patients and data about them).

## Multidisciplinary clinics: What we learn from each other

*By Sarah Preston*

In Canada, a visual tool has been created to help parents of new haemophilia patients identify bleeds that require treatment. This was created to assist and relieve anxiety in parents. The tool has a simple written booklet accompanied by a DVD showing bleeds, range of motion, signs you child may be showing limb favouring, what to do and how to do it. It uses voiceovers and gives lots of options and examples. The key message is "when in doubt, treat". The tool highlights the importance of support in newly diagnosed families.



Another important topic discussed was the importance of oral health. Patients with haemophilia and vWD have generally poorer oral health due to not trusting a dentist to understand their condition, having to travel long distances to a specialist, past mouth bleeds, access/waiting lists, history and myth of 'no local anaesthetics'. Ireland are conducting research around preventative oral care and have found that patients should have dental reviews no less than every two years. A psychologist can be used to deal with dental anxiety. Specialists need to write comprehensive letters (with contacts) for general dentists and decisions need to be made with the entire multi-disciplinary team. More oral health information needs to sent/given to the bleeding disorder community. There are no barriers to getting orthodontics, but the less invasive techniques should be employed for braces. We need to focus on preventative oral health care.

## Home treatments and self-infusion: strategies of delivery

*By Catriona Gordon*

Promoting and enhancing access to home treatment for people with haemophilia leads to improved clinical outcomes and quality of life, no matter what part of the world they live in. But both the benefits and the risks are magnified in developing countries.

Carmen Cunha Mello Rodrigues of Brazil cited studies indicating that home therapy results in 73% less absenteeism from school and work, 89% fewer hospital admissions, and 74% lower treatment costs. By drastically reducing the time between bleeding onset and treatment, joint damage and disability can be significantly diminished.

She also pointed out the drastic differences in the level of care in the developing world, noting that many people with bleeding disorders still receive no treatment at all and do not survive until adulthood. She described Brazil's "home dose" program, which gives patients one to three doses of factor concentrates for home infusion. This is particularly beneficial since most patients live many hours from the nearest treatment centre. However, recurring shortages of safe factor concentrates mean only about one-quarter of patients have access to the program.

The key to successful home therapy is clear, comprehensive, co-operative education tailored to each individual situation, said Bongi Mbele of South Africa and James Munn of the U.S. "It's important to consider the learners," Munn said. "If a person can't read, use pictures or videos... try to be as creative as possible so that the teaching resonates with the learners."

"Brazil's 'home dose' program...is particularly beneficial since most patients live many hours from the nearest treatment centre." Mbele stressed the importance of honest communication and understanding between the nurse/teacher and the patient. "If a person lives in a shack with no electricity, they have to find some other clean, well-lit place to infuse."

There are risks associated with home infusion. They include allergic reactions, infections, inhibitor development, and product contamination. Panellists agreed that ongoing education and open communication were key to meeting each of these potential challenges.

While peripheral venous access is the preferred route for infusion, there are many cases where venous access devices are necessary. Karin Lindvall of Sweden emphasized the need to train both parents and young patients to use and maintain the ports and recognize early signs of infection and other complications.

## Measuring Impairments in patients with haemophilia in everyday life

*By Cheryl Carnahan*

The first speaker of the session was Piet de Klein, a physiotherapist. He recommended 1-2 yearly measuring of all joints, e.g., the 50 metre walking test and the Figure 8 test (walking in a figure of 8 will test if the patient is favouring a knee). He mentioned the World Health Organisation (WHO) website which talks about working towards a common language for functioning, disability and health known as ICF (International Classification of Functioning Disability and Health) that provides a standard language and framework for the description of health. It is expected that ICF will become the world standard for disability data and social policy modelling and will be introduced in the legislation of many more countries.

ICF is WHO's framework for health and disability. It is the conceptual basis for the definition, measurement and policy formulations for health and disability. It is a universal classification of disability and health for use in health and health-related sectors.

The next speaker was Kate Khair from the UK, a nurse working at Great Ormond Hospital in London. She did a survey on the boys with haemophilia in her care. Most of the boys felt haemophilia was a burden, they wanted realistic treatment regimens and did not think they really had to treat in the holidays. One young man found out the hard way. Some couldn't be bothered to treat especially if they had never had a bleed. They often treated at the wrong time of day (e.g. after sport instead of before). She found that the parents had been educated about haemophilia when the boy was young but then the boy also needed to be re-educated.

The nurses role was support, education, "open ear" communication e.g. email, TXT, drawings etc. There was an increase in the quality of life due to rapid on demand and or prophylaxis but it was a burden according to the boys in the survey.

Dr Horacio Cariglic an orthopaedic surgeon from Buenos Aires spoke on measurements of impairments from an orthopaedic point of view. He concentrated on Quality of Life [QoL] and found that people with haemophilia had a lower QoL than the normal population. He said - accept your condition and know that it is treatable. Functional Independence scoring was discussed e.g. evaluation of everyday tasks e.g. toilet hygiene, bathing, eating, able to put both feet on the floor, walking, and distance able to be walked.

Sylvia von Mackensen a psychologist gave her point of view. She discussed the definition of health and the impact of impairment on daily functioning. She talked about the measurement of impairment and psychometric testing. She then discussed the several questionnaires that are used some of which were available in 34 different languages.

## Making the Case for Effective Clotting Factor Therapy in an Era of Health Technology Assessment

*By Richard Scott*

David Page (Canada) gave an introduction to **Health Technology Assessments**. Health technology assessment is cost benefit analysis of healthcare. In the global recession, restricted budgets are an increasing problem and there is a new focus on comparative economic benefits. Assessments look at short and long term outcomes and include medical, social, economic, and ethical issues while comparing treatments. Quality of life scoring system (QALY) comparing prophylaxis and on demand does show benefit but also has high cost. But other costs are ignored such as long term costs to society, cost to family, and cost of person unable to work. We need to be in the room when these cost assessments are being considered. *Resource: Key Concepts in Health Economics for Haemophilia Organisations, WFH.*

From **Cryo to Concentrates** was the theme of Triroj Krutvecho's (Thailand) talk and demonstrated how the cost of concentrates is the major risk for the development of treatment program. Thailand has only had recombinant factor available since 2006, however, factor concentrates from human plasma have been available since 1990. All treatment was in hospitals but only the rich could afford to buy the factor concentrates. As a result there has been inadequate supply, high mortality and morbidity. In 2006, the budget allowed for 4 vials a month per patient. The same number of vials was allocated to all types of haemophilia. Even with this small increase, the outcomes were that 90% of patients felt better and there was a 31% decrease in absences from school. Fifty-five percent felt treated like a normal person. Now vials are allocated based on need/severity. Even providing ¼ of what would be required for acceptable treatment made a huge difference to the haemophilia patients in Thailand.

Alok Srivastava (India) asked if **prophylaxis in children in the developing world** was possible. A critical component is the availability of factor concentrates. Often not enough factor concentrate is available to even treat bleeding episodes, much less consider prophylaxis. Only 25 countries in the world use prophylaxis or can afford to. In India, if prophylaxis was targeted only to young children (<5 years old) who bleed a lot then it may be possible. Bleeding problems are not evenly spread across the haemophilia population some bleed very rarely. Prophylaxis may be possible for the most severe cases for the first 5 years of life before switching to on demand treatment. Another problem is that only 25% of people with haemophilia are even diagnosed in most developing countries. If a patient develops inhibitors they cannot be treated anyway, as FVIIa is too expensive for India. If we are committed to prophylaxis then we must be committed to outcome analysis. Haemophilia joint health score and an ultra sound score can be used in the developing world.



Things are different, however, in the developed world. Marilyn Manco –Johnson (USA) spoke on **Prophylaxis in Children and Adults in the Developed World**. In young children cartilage is still under development. Children are prone to joint bleeds (knees, ankles) because there is less muscle development. An injury to cartilage whilst it is under development has more impact.

On the positive side, prophylaxis in children involves smaller doses due to their lower body weight. Children also seem to be more valued in society as priorities for health care. The goal of childhood prophylaxis is to maintain normal joints, but also to improve quality of life, reduce days in hospital/clinic, and encourage academic achievement. Measurement based on joint damage is not useful as joint damage is delayed by several years so by the time you can measure it is too late. Scale sensitivity is also a problem as it takes 6 years before the damage differences were apparent between cohorts. Clinical bleeding episodes do not match well to MRI scan results. A child may have 0 clinical bleeds but have significant changes on MRI scan. Bleed counts are not a predictor of outcomes. Individuals have very different synovitis reactions to bleeding into joints.

Secondary Prophylaxis in adults with recurrent bleeds has been effective in controlling bleeds.

The education and knowledge about treatment, and the aggression of treatment has improved outcomes. Physiotherapy has also improved outcomes. Joints bleeds are reducing and target joints are reducing across all individuals whether on demand or on prophylaxis.

## Helping Children Cope with Bleeding Disorders

*By Lorraine Porter-Bishop*

Cesar Garrio (Venezuela) gave a father's prospective on having a son with severe haemophilia. Cesar talked about the why me! Why my son? What do I do! feelings that come with diagnosis. Cesar set about learning about the disease looking for available medicines, looking into the safety of available products, and learning about life expectations and clearing the doubt he once had. He discussed getting the child involved in understanding about their condition and how to transfer parent's knowledge to their son. Cesar discussed the comprehensive care which is now available. He went through a process from building a defence around his son, to looking at appropriate, comprehensive care with the medicine available and safety of products available. His goal was looking at quality of life.

Gordana Stevanovic (Serbia) gave a mother's perspective and gave an account of the enormous stress mothers deal with when they get the news they have a child with a bleeding disorder. She believed more knowledge helps build positive attitudes long term. Building awareness will help with self-confidence; however, it needs to be translated into a age-appropriate language for the child to be able to understand. It was also important to prevent the child feeling guilty or ashamed about their condition.

Christine Keilback (Canada) gave an overview of the Canadian Hemophilia Society and showed slides of their family camps. These are similar to camps held in New Zealand. Christine felt these camps gave families the opportunity to discuss any issues in a relaxed environment. Many families became very close as they shared their experiences with living with haemophilia.

Ed Kuebler (USA) gave a psychologist perspective. Ed talked about the coping skills of children and how they use their thinking process of thoughts, feelings and emotions. He discussed the self prospection defence. Ed said we need to be teaching effective coping skills – learn effective skills to deal with difficult situations. He suggested this website for further information: **http://copingskills4kids.net**.

## Sports: Dangerous or Beneficial

*By Catriona Gordon*

Do children with haemophilia need to participate in sports? What are the limits? What are the benefits? During a Monday morning concurrent session, physiotherapists and physicians discussed some of the pros and cons of sports activities for children with bleeding disorders.

While participation in sports can help build physical strength, improve musculoskeletal structure, and increase self-esteem and a sense of belonging, deciding to engage in sports can be difficult for children with bleeding disorders and their families, who must weigh the risks and benefits carefully.

Sports can be classified in three categories, said Andrés Thomas, a physiotherapist at the Fundación para la Hemofilia, Argentina: those that can be safely recommended, those whose relative risks and benefits must be assessed for each individual, and those - like boxing, rugby, or American football - whose risks outweigh the benefits, even for people without haemophilia. Thomas said families should consult with professionals while their children are young to find the sport that best fits their circumstances.

Physiotherapist Nichan Zourikian of Montreal's Sainte-Justine Hospital, Canada, said the recommendations are not the same for every patient. "Haemophilia is a chronic condition, and expectations are different in each age group," he said. Younger children need to make friends; adolescents have other interests; and adults may need the cardiovascular benefits of sports.

Dr. Sylvia von Mackensen, a medical psychologist at the Institute of Medical Psychology of the University Medical Centre in Hamburg, Germany, showed the results of three studies assessing the impact of sports in children's quality of life. The children's own perceptions their physical activity significantly affected their health quality of life (HQL) indexes, she said.

Martial de Haro, an expert scuba diver and haemophilia patient from France, said, "One should know and respect his own limits, stick to treatment, and overcome fears."

## Young Voices

*By Lynley Scott*

New Zealand's own Deon York discussed **How to Recruit Young Leaders in Haemophilia**. As always, it is great to hear Deon speak and he represents HFNZ so well. Deon commenced this session by outlining the types of leadership and the challenges that are associated with leadership – attracting and retaining; developing necessary skills; retaining institutional memory and continuity; and time and money of organization and individual. It was important to keep in mind who you want, who your target audience and what leadership skills are needed and what can be developed.

When recruiting, there are a number of pitfalls including:

- Recruiting for a lifetime, instead of recruiting for short-term project teams,
- Expecting announcements to get volunteers rather than personally approaching people,
- Doing 'it' alone,
- Assuming "no" means "never",
- Believing it is better to fill a position than leave it vacant and wait for an appropriate person,
- And being position-focused rather than person-focused.

When trying to recruit future leaders, it is important to look at what is important to Generation Y (1978-1994). In order to retain young leaders, NMOs need to mentor their younger volunteers with more experienced ones (this also assists in retaining experience), encourage development in the good and the bad times, and work on a shared ownership in the organisation. It is also important to see what young leaders can gain from becoming involved: educational opportunities, professional experience, increased confidence, opportunity to work in a team and develop these skills, opportunity to work with a diverse group of people, and shared ownership of the NMO. Likewise it is important for the NMO to see what the organisation can gain from developing young leaders: fresh perspective, injection of new skills, energy and enthusiasm, and opportunity to share knowledge and grow programs.

Deon gave examples of how HFNZ are trying to develop their young leaders: embracing social media, developing specific youth roles, promoting education, targeted leadership development, and having an aspiration/goal of the organization that can inspire people. He also discussed how his involvement had developed over the years and how he had strong mentors who didn't give him all the expectations and responsibilities at once, rather gradually. He believed in HFNZ's mission and saw the value in long term commitment.

In conclusion, Deon outlined some questions; organisations must ask in order to develop future leaders:

- What leadership will the organisation need?
- What are the potential challenges?
- Where will the future leaders come from? Who are they?
- How can I recruit and retain them for a sustained period so skills development is possible?

Jacob Beck Andersen (Denmark) spoke about **Closed and Open Electronic Forms**. Jacob began this session by discussing the Danish Haemophilia Society. He then discussed why electronic forums are important – including provide opportunities to share experiences, create important networks, can provide opportunities for NMOs to keep their members up to date, and can potentially recruit new, especially young, members. There is a role for both closed and open forums and while there is no one that is more appropriate, it is dependent upon the situation. At WFH Congress in 2006, Los Bleedos was launched as a closed forum for people with bleeding disorders. It was set up by 5 youth and while initially successful, it appears that it was overrun by Facebook and Twitter. So is Facebook the solution to this – No. Facebook has huge positives but can also offer some challenges, including the lack of filter when a question is asked, lack of privacy and confidentiality, too many forums, and the possibility of members becoming targets of drug or insurance companies. Besides all the other known problems. Jacob discussed a case recently when a person with a bleeding disorder died in a Danish hospital as doctors perceived him as being intoxicated and did not believe he had a bleeding disorder. Within 10 hours, comments and discussions appeared on Facebook around the medical care and safety of people with bleeding disorders.

Jacob concluded that:

- Open and closed forums belong to the future.
- They are a good way of involving the next generation.
- The NMO and user must know the positive and negative consequences of using them.
- Have a clear line between open and closed forums. Do not use all closed forums.
- A mix between Facebook and other forums is ideal.

In the future, the Danish NMO is going to strive to be more active and aware of new media forums, and will strive to develop new ways in which to integrate this into their work. Jacob felt that while not all forums should be closed, that a new closed and moderated forum is taking shape in Denmark.

Marika Jacobsen (Sweden) then spoke about **How to Form a Youth Group and What Activities Create Success**. In this session, Marika and her fellow presenter discussed how the Swedish Youth had formed a Youth Group within their NMO. The Swedish NMO has approx 1005 general members in the country, and approximately 25 members of their youth group (between 16-26 years). The aim of the youth group is to give its members a sense of belonging, unity, friendship, shared interest and a chance to discuss life with fellow members. The youth group started in 1991 with no budget; in 1994 the group was given a set annual budget. Interestingly they felt this has made the group more successful as it has given them more of an ease to plan activities and be involved. They provide gatherings 3 times a year (Autumn, Winter, and Spring) and most communication is via email. Gatherings are usually held over a weekend, including one nights' accommodation away and can include anything from bowling, go-karting,, skiing, and going to a health spa, usually their winter event is a skiing trip. During the weekend they will include discussions, address questions, and make plans for the next event. They include participation from medical staff to provide education. They have found that by providing this youth group, youth have found there to be many positives including widening their social network, acceptance, and advice from others (job, education, treatment, etc.)

## Therapeutic training for haemophiliac children

*By Michael Ho*

Most of the sessions I've attended were dedicated to the ageing issues, but I have also attended couple of sessions on teens and family.

The session titled **Therapeutic training for haemophiliac children** outlined the use of social camps in France to introduce children to sporting activities that they would otherwise never experience and provide a peer environment

where children can feel comfortable to discuss their needs. It is much easier to educate children when the education is slipped in between fun activities. HFNZ has very successful used this strategy with the camps for different targeted groups for many years.

The other session related to the psycho-social side of a haemophilic child. Each child has their own perception of what haemophilia is - it is a totally subjective experience. With prophylaxis, their association with haemophilia is really the 'treatment' itself, as most of them live such a 'normal' life without any the complications experienced by previous generations. The treatment experience can be treated objectively or subjectively.

Some adolescents have treatment compliance issues, for example they only visit a treatment centre for emergency and some exhibit dangerous behaviours. Some of the reasons given for the resistance are:

- Having the take responsibility – mum used to do it
- Difficulties – distance, cost, transport, work commitment and time, etc
- Judging effectiveness, they think they have done enough
- Disbelief in the treatment
- Not managing to face the demands and difficulties of the treatment alone

Self-management is the ultimate goal for people living with a chronic illness. It has been shown to be strongly linked with healthy outcomes and increased quality of life. Self-management refers to the individual's ability to manage the symptoms, treatment, physical and psychosocial consequences of lifestyle changes inherent in living with a chronic condition.

Website resource:
**http://patienteducation.stanford.edu/programs/**

The nurses' role in self management is to provide education and supportive interventions which will give patients the knowledge, self confidence and motivation to manage their chronic condition.

The goals of patients are to adhere to the treatment regimes, the identification and prompt treatment of bleeding episodes, knowing when to seek advice, attending appointments, adopting a healthy lifestyle and managing additional therapy such as HIV or HCV treatments.

The positive outcomes of self-management are reduced acute admissions, reduced absenteeism from school or work, and reduction in healthcare costs. It also increased self confidence, health, wellbeing and independence. It empowers the patients to live positively and increases compliance with treatment regimes.

Potential barriers to self managements are excessive dependency, emotional immaturity, social isolation, lack of formal education, stigma and multiple chronic conditions. Individual difficulties are anxiety, being overly preoccupied with condition, depressive disorder, psychological problem, and the demands of growing up.

## Managing haemophilia through infancy into young adulthood

*By Chantal Lauzon*

During the World Congress there were many Industry Symposiums sponsored by manufacturers of clotting factors that provided forums for discussion on certain topics such as inhibitors or prophylaxis. One such symposium, sponsored by Pfizer, concentrated managing haemophilia at different stages of childhood and adolescence.

**Initiating therapy in childhood**

As 50-70% of people with severe haemophilia are diagnosed with the first 6 months of life, Jorge DiPaola, a haematologist from Argentina who now works in the USA, spoke about initiating haemophilia treatment.

Around 4% of people are diagnosed following an intracranial bleed at birth (ICH). The mode of delivery (vaginal or caesarean) does not appear to affect incidence of ICH but difficult delivery deliveries (forceps or vacuum-assisted) can cause problems. He recommends imaging to check for ICH in all cases of traumatic delivery in children with a family history of a bleeding disorder. DiPaola also did not recommend circumcision, but if parents of babies with severe haemophilia choose to have them done for cultural or religious reasons then he recommends they be done by an experienced surgeon under factor cover.

It is thought that the development of inhibitors can be related to early treatment and inflammatory processes in early childhood. Although the data is not clear, it appears that the intensity of treatment may be more of issues that which product (recombinant or plasma-derived) is used.

Primary prophylaxis is known to prevent bleeding, prevent joint disease and improve quality of life and attendance at school. The disadvantages are only the time it takes and the high cost. DiPaola recommends starting prophylaxis immediately if a baby has an ICH or has 3-4 bleeds in their first year. The dosage depends on several factors and the decision to start must be decided with the family and the health care team as it involves a serious commitment from all, especially considering the difficulty of venous access in babies. In the USA, 60% of children with severe haemophilia are on prophylaxis by age 6-10 years; in Canada it is closer to 80%.

It has been difficult to systematically evaluate the impact of vaccinations on inhibitor development. All regular childhood vaccinations are still recommended, but DiPaola suggested delaying for a few weeks if a child has an active bleed at the time they are meant to be vaccinated so as not to overload the immune system.

**Personalising therapy for the growing child**

Although it is well established that prophylaxis can prevent bleeds and joint damage, Kathlijn Fisher (The Netherlands) explained how due to the variety of individual bleeding patterns, both the dose and the dose interval are increasingly being tailored for each child and should be constantly adjusted as the child grows.

According to the Dutch prophylaxis regime prophylaxis is started after the first joint bleed, and the dose/interval escalated in case of bleeding. Prophylaxis is stopped if the dose is low and bleeds are rare (as child ages).

**Figure 5.**
***Factors that influence bleeding in people with haemophilia***


Several factors influence bleeding a growing child with haemophilia and the prophylaxis regime needed.

**• Bleeding Phenotype**

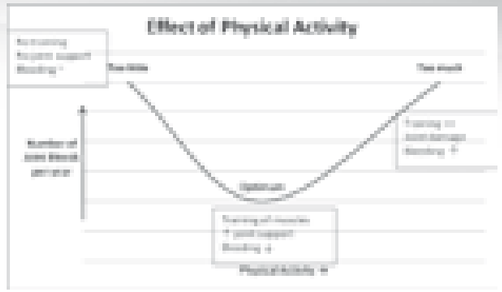
Even in people with severe haemophilia, there is a wide variety in bleeding patterns, i.e. some seem to have lots of spontaneous bleeds and others hardly any. The bleeding phenotype can mean the some people can stop prophylaxis at 21 years, while other need to continue. The age at first joint bleed is the best predictor of phenotype (the earlier the first bleed, the more severe the phenotype).

**• Age**

The number of joint bleeds a child experiences increase with age and then tend to level out in adolescence and adulthood.

**• Physical activity**

Children with haemophilia in developed countries tend to be very active. In the Netherlands, most do sports up to five times a week, often the same types of activities as 'healthy' children, with the exception of hockey which is not played by children with haemophilia. Some physical activity is highly recommended to build muscles and stability around joints, however too much can cause damage and increase bleeding. It is very important to protect ankles as they are the most affected joint in haemophilia and most joint bleeds in ankles occur during the school years.




**• Half-life**

FVIII half-life increases with age (over age 10 years vs. younger). This makes a difference during prophylaxis as with a longer half-life you get the same protection with less frequent infusions, so less of a burden for the patient.

**• Dose**

Breakthrough bleeding can often be reduced with a higher dose of prophylaxis. Fisher commented that if a person with haemophilia is going to be really active or a professional athlete then they should consider taking low dose prophylaxis every day, not regular high-dose alternate-day prophylaxis and then extra for sports training.

The high cost associated with high clotting factor consumption is prohibitive for prophylaxis in many countries. Adjusting dose to the bleeding pattern and activity level of patients, as is bring practised in the Netherlands, Sweden and Canada, may prove a very important step in increasing the efficacy of this expensive treatment without jeopardising outcomes.

**Challenges and solutions in managing the adolescent and young adult**

Kate Khair (UK) discussed how haemophilia is a family disease and the different challenges faced by a teenager with haemophilia and their family. Initially children are reliant on parents and there is often a parental reluctance to 'let go' as a child grows up. It is important to educate parents about haemophilia so that they are fully informed and it can be accepted within the family.

Adolescence is a time of transitions, physiological, hormonal and emotional. Some teenagers find it very difficult to accept limitations imposed by haemophilia.

The transition from care delivered by parents to self-administered care in the adolescent and young adult requires careful planning with an individualised approach to educating and supporting boys to be responsible for their treatment. Khair suggested that the idea of self-infusion needs to start to be introduced at around age 6-8 years because by age 13 youth can be unengaged and non-compliant. She thinks that self-infusion should be mastered by around age 11-12 years for best results. An individualised approach is needed however that involves the parents and where there are no rights or wrong. Educating young people about haemophilia and its consequences is important.

A 'concordance' rather than a 'compliance' approach to prophylaxis should be taken with adolescents with haemophilia. Complete compliance with prophylaxis regimes might be unrealistic, as is the idea of life-long primary prophylaxis (due to cost, venous access and necessity). Prophylaxis should be tailored to the individual and to their life. If it fits their life, then teens are more likely to treat regularly. For example, if weekend nights are a much for active time then school days, consider having prophylaxis in the evening on Fridays instead of the morning. An honest and open relationship between the teen and their parents and the haemophilia centre is needed.

Key principles of concordance approach:

Work collaboratively with the adolescent with haemophilia (discuss activities and what time of day would suit them best, etc.)

Emphasise personal choice and responsibility

Focus on their concerns about treatment.

Products and life are constantly changing to the healthcare team, patients and parents need to remain flexible.

# Adults & Ageing

## Aspects of Ageing

*By Michael Ho*

It is great to see the new emphasis on mature people with haemophilia (PWH) in this congress; there were wide range of topics covering age-related issues. With the advance in treatments, the life expectancy of a person with bleeding disorders has increased dramatically, almost matching that of the general population. With the increasing number of PWH living a much longer life, a new set of age-related issues are being recognised for the first time in history.

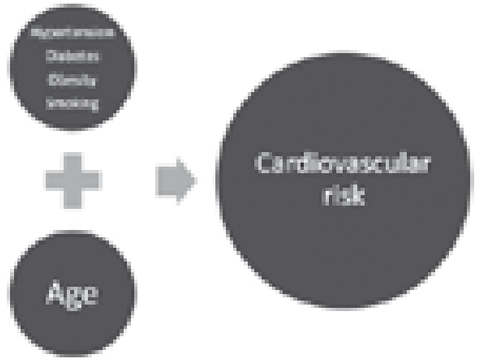
The availability of safe and effective factor concentrate, prophylaxis and comprehensive care leads to much longer life expectancy in PWH. There is an emergence of a middle-aged and elderly population. Although 77% of seniors over the age of 65 years have two or more chronic conditions, there is little experience of managing concurrent medical problems in individuals with haemophilia in this age group.

**Does haemophilia protect against cardiovascular/heart disease?**

The findings are unclear as there is lack of specific information on cardiovascular disease in haemophilia. Lack of systematic reporting, methodological issues, poorly characterised study groups and small samples cause concerns on the reliability of the collected data.

The current indicators are PWH have the same risk as the general population in developing cardiovascular disease.

**Figure 6**. *Cardiovascular risk factors*




With the reduced mobility of mature PWH, there is an elevated risk of obesity leading to hypertension and diabetes. The clinical picture of haemophilia has dominated by bleeding, infectious disease and musculoskeletal disease. Much of the medical care was delivered by Haemophilia Treatment Centres (HTCs). Who is checking blood pressure, proteinuria, screening for diabetes, bowel and prostate cancer etc?

**Orthopaedic issues**

Haemophilic arthropathy may be complicated by degenerative joint disease.

By 2030, in the general population:

- 1st total knee replacements will increase by 673%
- 1st total hip replacements will increase by 174%

The challenge of managing joint disease may remain a crucial part of comprehensive care, but the nature of care may change to include more rehabilitation and different specialists.

**Osteoporosis in haemophilia**

Several studies have noted an increased risk for osteoporosis in males with haemophilia. The risk factors are: age, liver disease, immobility, haemophilic arthropathy and low body mass index. Ongoing studies will assess the role of screening and intervention in haemophilia.

To help decrease the risk comprehensive care should include more physical therapy and rehabilitation therapy targeted at older individuals. In addition to reducing the risk of osteoporosis, these measures would also potentially reduce the impact of joint disease, and improve balance, coordination and movement.

The success of high quality treatment for haemophilia has resulted in an increase in life expectancy and an increasing population of older individuals with haemophilia. This is a major triumph in clinical care and therapeutic development. There will be new challenges for HTCs to ensure that PWH receive the standards of care available to prevent age-related disease.

Currently, we do not have total 'Comprehensive Care' in New Zealand for PWH. HTCs must collaborate closely with other medical teams, and act as a hub to steer PWH through the different primary care specialists. Ultimately, however, a PWH must take an active role in liaising with the various health care providers with the aid of the HTCs.

## Ageing and haemophilia

*By Grant Hook*

It is very positive that we are now having new generations of PWH with the average age of dying now 65-70 years up from the age of 10 in 1957. This of course brings new challenges for PWH and their health care professionals.

PWH have the same ageing issues as the rest of the population but with added complications. With the high cost of visiting the doctor many patients only medical contact is with their haemophilia treatment centres. It is very important to also visit your GP/primary health provider, who is used to carrying out routine medical checks for monitoring blood pressure, diabetes and other age-related complications that haematologists do not monitor.

As always it is important for the patient to take responsibility for their own health, it is up to you to monitor and make sure that regular health checks happen and that any issues are followed up.

With age there are increased risks of poor balance and associated falls. Oral health is also critical as this can lead to many health complications if not monitored. Many mature PWH have joint damage and there was a discussion as to the merits of prophylaxis in older PWH, with studies now looking into the benefits and disadvantages of this.

At the extreme end, there are the issues around what to do once we reach the old folks home such as: How will our care be administered? Who will be responsible for care if we get Alzheimer'? How will health care professionals manage this?

The recognition of these ageing issues had increased at the conference with many more sessions now related to ageing and a greatly increased awareness worldwide of the increasing care required with planning and studies into how this will be achieved.

## Meet the experts - Orthopaedic Issues

*By Michael Ho*

Mauricio Silva discussed the issue of blood inside joints. Even short exposure to blood affects the cartilage. His view is to aggressively remove blood from acute joint bleeds. He supports aspiration of all joint bleeds as there is only a small amount of tolerance before long term degenerative joint damage sets in. The longer the blood is inside the joint, the more damage it cause.

The discussion moved on to surgery. Thorough assessment is required for any proposed surgery, taking into consideration of the state of other joints. Pre-op planning is very important. Some UK surgeons believe that you shouldn't do surgery on two joints in the lower limbs together, but working on the shoulder and the elbow at the same time is ok; but it's up to individual surgeons to decide.

A question was asked about post surgery sexual activity. The experts said "Just do it!" however, because damaged joints do cause issues, clear communication is crucial. The experts suggested experimenting with different positions to avoid bleeding. Target joints vary per individual and it important to find positions that minimise stress on those joints.

Some study shows that 83% of joint replacements in PWH are still in place after 10 years, some often lasting 15 to 20 years.

Instead of replacement, some patients opt for joint fusions (arthrodesis). The primary reason to the fusion is to relieve pain. Fusion of the ankle joint right below the leg bone removes the pain permanently as there are no more bleeds into the joint to cause pain. The front joint in the foot retains motion, but specialised footwear may be required, i.e., shoes with soft flexible soles to compensate for the restricted motion. The long term risk of arthrodesis is immobilisation which could cause muscle and strength loss and joint position may affect gait.

In developed countries, people on prophalyxis have minimal joint damage and are therefore less able to recognise a bleed. **Physiotherapy is extremely important, as people don't often manage their bleed properly**. In some developing countries, physiotherapy is the only form of treatment available because they do not have any factor replacement therapy available. With the lack of

prophylaxis, they often develop joint issues early which lead to target joints. Physiotherapist should concentrate on checking real joint movement and individualising treatment.

After a total knee replacement, it was recommended that physiotherapy be started within 24 to 48 hours. Range of movement exercise should be introduced within two days. Patient should use a walking aid and exercise as soon as the pain goes away.

Mauricio Silva stressed that **there are no spontaneous bleeds, there is always a reason for a bleed**.
Early education leads to early management and early rehabilitation.

There is also no such thing as the best sport for PWH; activities should match the physical abilities of the individual. Activities such as golf, fishing and cycling are low risk, but they may still pose challenges to some patients; i.e. golfing and fishing could be hard on the elbows, while walking on uneven terrain could be hard for others. One must take into consideration the frequency of the activity and type of potential injuries. In choosing a sport, a family must consider the interest of the child, and what type of coaching is available locally. Attention should be paid to the level of joint damage and the potential for further injuries. Swimming is a very low impact activity which gives a total body workout without stress on joints. Non-weight bearing exercises do not help increase bone density.

**http://www.wfh.org/2/docs/Publications/General_Guides/Exercise_Guide_med.pdf**

## Ageing - what the other experts said

*By Mike Carnahan*

The Danish Haemophilia Society has carried out a study ***"A Long Life with Haemophilia"*** and found:

In Denmark PWH are living longer but are experiencing major physical problems including pain, reduced strength and impaired movement from joint damage, complications from older age, and are concerned and worried about their futures. Getting help from others and government is becoming more and more difficult. Their standard of health was poorer than the age related general population, especially with respect to pain. Their frequency of bleeding incidence had not improved compared to an earlier 1988 study. I in 5 had HIV and 1 in 2 had HCV.

This older group was found to have a higher education than that found in the general population. About 1/3 were living alone making life even more difficult. There was a trend towards early retirement. A variety of equipment was used to assist daily living. Only 1:5 reported living a normal life.

Professor Elizabeth Steinhagen-Thiessen from Berlin, Germany, noted people with haemophilia are now living longer and as a result, growing numbers are developing age-related conditions such as cardiovascular disease, cancer (influenced by HCV and consequent cancer of the liver), arthritis, osteoporosis, hypertension, renal disease and obesity. Agents used for treating these diseases in the general population are often not suited to the patient with haemophilia. Then there are issues such as impairment to hearing, eyesight, muscular strength, mobility and flexibility. By incorporating the geriatric team into the haemophilia team a "Geriatric Assessment" can evaluate all these issues, hopefully early enough to detect underperforming attributes and carry out restorative or salvage steps to maximize functionality and enhance daily living.

These measures, however, cannot replace the practice of lifelong preventive health care, making continuing access to care on the basis of need fundamental to a good health system.

Dr Jerry Nolan of Nottingham pointed out haemophilia care over the past 30 years has been dominated by management of individual bleeding episodes, coping with virus transmission, and, more recently, embarking on joint replacement. Men approaching older age have therefore probably given little thought or energy to dealing with health care prevention for older age. Dr Nolan questioned who in the health system is monitoring issues such as blood pressure, diabetes, and blood results? He also noted the role of prophylaxis in adults is unclear but he clearly pointed out the benefits of physiotherapy as part of comprehensive care, especially with regard to balance coordination and movement. Dr Nolan produced a very interesting slide of F VIII usage in various age bands which showed a marked drop in factor consumption after the age of 65 years.

**New challenges facing the aging PWH population**

Zygmunt Gruszka of the Swedish Haemophilia Society noted that thanks to treatment with factor concentrates and despite the catastrophe of HIV and hepatitis C, Sweden is experiencing its' first generation of elderly PWH, and the number of senior PWH is increasing. This creates new challenges not only for the haemophilia care centres (HCC) but also for national haemophilia societies (NHS). Today we have a only few results of studies reflecting the health status and quality of life among senior PWH. However, almost nothing is done to enlighten correlation between patient expectations and what the HCCs can or may offer when other diseases, not directly resulting from haemophilia, are to be considered and treated.

Therefore does the new situation require a new organisation of the HCC or diversity of associated physicians, new tests to be regularly performed? The answer is: yes!

The new challenges for NHS's is to formulate overall strategies and platforms for actions/lobbying to meet the expectations expressed by elderly PWH. As a start, the NHSs need to collect their own data about the current social, economical, and health status of their senior members, and discover its expectations of future demands and needs, possible fears, threats, etc. The Swedish NHS addresses the overall problems regarding accessibility for and discrimination against disabled people in alliance with other disabled people's organisations, while specific issues concerning care of PWH, including, elderly PWH, are addressed within the advisory councils.

**Medical aspects of ageing and wellness in people with bleeding disorders**

Dr Alison Street of The Alfred Hospital, Melbourne identified the advent of safer and more abundant supplies of factor replacement therapies, together with the development of centralised treatment in comprehensive care facilities, as the catalyst for an increased requirement for management of an older PWH.

While severe FVIII deficiency may partially protect an individual from coronary artery disease, the morbidities of ageing pay no respect to haemophilia and occur as in the general population. Recently Konkle, *et al.*, published recommendations for managing co-existent medical conditions in persons with haemophilia (PWH) including arterial disease, kidney disease, and cancer, as well as for conditions over-represented in the haemophilia community such as joint disease and liver disease. The authors emphasise that there have been no randomised clinical trials performed in PWH and thus their recommendations are weak according to ''rules of evidence'', and based on extension of studies published on non-haemophilia subjects. Many medical procedures, undertaken with the aims of diagnosis and treatment, have been successfully performed in PWH. Communication between all clinicians involved in a patient's care is critical, as is the understanding and engagement of the patient. Along with supervision of care of established disease, there are many opportunities for haemophilia clinicians to encourage ''wellness'' in their patients. They can facilitate structured individual exercise and falls and balance programs, attention to diet, cessation of smoking, moderation of alcohol intake, psychological support, and linkages to primary care physicians and geriatricians. There is much benefit in the involvement of PWH in social and mutual support networks such as haemophilia organisations.

**Healthy techniques**

Angela Forsyth a physiotherapist from Philadelphia USA commenced by pointing out musculoskeletal bleeding as the most common type of bleeding in haemophilia and over the years, this results in chronic synovitis and arthropathy, leading ultimately to joint destruction. As the population with haemophilia ages, the problems caused by these complications become more evident and impact quality of life. Individuals are faced with chronic pain, difficulty with functional mobility, and challenges in performing everyday tasks as well as participating in physical activity at work, home and recreationally. Physiotherapists working with these individuals focus on addressing the complications of musculoskeletal bleeding. As the joints become damaged through bleeding, many other challenges arise. There are typically problems with proprioception (the ability to sense the position and location and orientation and movement of the body) and balance that can lead to increased falls. Muscle weakness, muscle contracture, and atrophy are a concern. In many cases, damaged joint surfaces lead to bony destruction and joints that are painful and often unstable. The presence of deficits in these areas leads to the individual having difficulty in being actively able to participate in everyday life. Interventions by physiotherapists are performed as part of conservative management and in coordination with joint surgeries, when that option is available. Physiotherapists seek to support the individual with haemophilia by maximising the functional capability within the limitations present. Strategies for remaining active and a personalised fitness program can be recommended. Supportive braces, shoes, and mobility aides may be prescribed. Assistive devices and tools may be suggested to assist in performing activities of everyday living. A home assessment can also be performed to maximise safety and help prevent falls.

**Ageing and haemophilia – personal experience in a developed country**

Mike Carnahan of the Haemophilia Foundation of New Zealand canvassed firstly the issue of haemophilia, saying he probably experiences fewer problems from haemophilia than he did as a child or adolescent. However, the consequences of haemophilia were having a marked impact. He noted the 9th Haemophilia Consortium Meeting in Brussels March 2010 concluded "co-morbidities will become a bigger problem than haemophilia itself". These problems included haemophilia arthropathy, chronic synovitis, degenerative arthritis all contributing to chronic pain and acute pain and resulting in most joints not working properly thus creating limitations to daily activities and daily life.

Then there are the diseases of ageing. While PWH seem to have some protection from cardiovascular disease, we all have to deal with higher than usual rates of hypertension and diabetes, maybe HIV and its debilitating palliative treatment for 20 odd years. Probably 90% have contracted HCV or HBV and endured the treatment of up to 48 weeks with 50% retaining chronic HCV. Cancer is common in conjunction with haemophilia due to HCV, with rates of disease greater than general population. Renal Disease is also common in conjunction with older PWH. In addition to joint disease arising from haemophilia we also have the usual joint disease associated with older age.

We are probably the group that will be most severely affected by joint and muscle damage as we are from the era of no treatment during our childhood and adolescence. He suggested this group needs to deal with the consequences of haemophilia and getting older by taking control of overall health status. This implies each individual accepting responsibility for their health and becoming proactive:

- Be informed
- Monitor and manage signs and symptoms
- The impact of haemophilia can be managed using home therapy, and adhering to prophylaxis if prescribed
- Keeping to treatment protocols - taking medications as prescribed especially anti-inflammatories and pain relief
- Retaining functionality (doing the physiotherapy exercises)

- Carefully managing veins
- Ensuring orthotics are maintained
- Break the day up with activities and rest periods
- Staying in touch with HFNZ
- Using public preventive screening programmes such as for prostate examination
- Get any restorative surgery done as early as possible. Use arthroscopy, synovectomy, arthrodesis, TJR, etc.
- Deal with joints that are painful and restrictive especially on mobility
- Regularly report health status
- Achieving ideal body weight
- Giving up smoking
- Avoid alcohol
- Modifying diet – i.e., low cholesterol
- Don't abdicate control of your health to the health system

It is no surprise that age is a strong predictor of quality of life [QOL] – as one becomes older so QOL tends to diminish. Researchers have identified PWH severe, generally recorded poorer levels of QOL than the general population. Issues that were found to be strong predictors of QOL were age, severe haemophilia, and chronic HCV and are markedly affected by physical functioning, hence the need to accept responsibility for your health and becoming proactive.

In the USA only 2% of people with haemophilia are over 60 years. NZ presently has 9% of PWH + 65 yrs, a statistic that will increase to 23% in 30 years time. In UK median age for HIV-neg PWH severe is 63 years. In NZ over the last 5 years the average age at death has been 63 years over all PWH. So NZ has plenty of room for improvement.

## Pain management and coping

*By Grant Hook*

Most, if not all, PWH will experience some level of pain. If it lasts longer than 3 months then it is classed as chronic pain. Most pain medication is prescribed by the patient's haemophilia centre with very few patients worldwide ever having a consultation with a pain specialist.

Pain management is a relatively new area of medicine that is still developing assessment tools and guidelines. Many health professionals rely on patient's definitions of their pain levels when prescribing medications. There is a need for a clear definition of chronic pain and more evidence-based guidelines for treating pain.

There was a very good presentation by a haemophilia nurse of how her hospital worked with patients:

- They use the guideline of pain for more than 3 months is classed as chronic.
- Signs of chronic pain are increased requests for pain medication and patients seeking multiple prescriptions from doctors, increased requests for medical certificates, more visits to the centre/emotional dependence and increased product use.
- Following these indicators the patient is triaged by a nurse and referred to appropriate health professionals, such as a rheumatologist, physiotherapist, orthopaedic surgeon, counsellor, and dietician.
- Questions to be addressed include: is the bleed real or pain? Patients are given pain meds and if the pain is not resolved they go through the process again. At this point they do have access to a pain management specialist to refer patients to.

The old adage of "if in doubt treat" needs to be questioned. Is this true with a patient on prophylaxis? Treating any pain is a waste of product. What could be saved if we were to teach more PWH about pain and when and what to treat?

People with persistent pain often have a high disability level. They cycle through periods of over- and under- doing it and think they need rest which is used to avoid activities. They believe they need medication to deal with the pain when in some cases further investigations will lead to a diagnosis that will resolve the pain. Patients wish for more sleep to enable them to do more activities. They generally want more mobility and diagnoses to explain and give credentials to their situation.

There is increased recognition that a comprehensive pain management programme improves patients QOL through the ability to use techniques to recognise chronic and real pain and how to deal with it.

## Haemophilic Arthropathy (joint disease)

*By Chantal Lauzon*

**Figure 7.** The ankle joint and associated ligaments. The numbers indicate the three regions that need to be examined to assess arthropathy in a person with haemophilia.

Despite good prophylaxis, people with haemophilia still suffer from joints bleeds resulting in joint damage. Monique Van Veegeren (Netherlands) gave a detailed presentation on the effect bleeding has on joints. Essentially, haemophilia arthropathy (joint disease) has two components: inflammation of synovium (joint lining) and cartilage damage. Blood exposure causes the synovium to become inflamed and this contributes to joint tissue damage as well as causing cartilage damage independent of the synovium. Studies have shown that exposing joint tissues to blood for more than two days can cause long-term damage.

The WFH operates a Muscolo-Skeletal (MSK) Committee, a group of surgeons and physiotherapists who work to promote research and training to improve the orthopaedic care of people with haemophilia. Pier Luigi Solimeno(Italy) suggested that as a person ages haemophilia can really be considered an orthopaedic disease with bleeding complications instead of the reverse. Solimeno gave an update on the interests and activities of the MSK Committee. The resources available to a local region dictate the standard of care available, but the role of the Committee is to educate surgeons, physiotherapist, and nurses with the best information and techniques possible for their resources. A specific haemophilia joint score has now been developed. They are and have developed a number of guidelines (i.e. on management of knee replacements) but need still research in a number of areas (inhibitor patients, ankle replacement, revision surgery,etc.)

Finally, Adolf Llnàs (Columbia) spoke specifically about the ankle joint. Ankles are subject to repetitive bleeding which often leads to deterioration of joint function. Ankle arthropathy is disabling as it modifies posture and gait. Llnàs believes clinicians and physiotherapist need to look at ankles problems as multi-component problems and need to address each.

As marked in Figure 7, the first problem is due to the shortening of the gastro-soleus (lower calf) muscles (1). This problem is often overlooked and is the result of muscle bleeds into the calf. Calves are a high risk area for bleeds because they have four well defined compartments but they are also difficult to examine and treat. Treatment options include lengthening the gastro-soleus muscles and Achilles tendon through physiotherapy; however, if the damage is long-established surgery might be needed.

The second problem involves the actual ankle joint, the area where the tibia and talus connect (2). There are three stages to damage in this area, synovial activation (swelling), deterioration of the cartilage or bone, and anterior impingement (when ligaments or tendons in front of the ankle become trapped between the bones). Swelling affects range of motion, muscle strength and joint speed. The swelling of the synovium must be addressed first to return the ankle to a state where prophylaxis is effective. If there is constant activation then a synecvectomy (radio or laproscopic) might be effective. Anterior impingement results in loss of flexion because the bones are jammed together where there is no longer cartilage. To help, surgery can be done to shave the bone, thereby increasing range of motion and reducing further bleeding. The deterioration of cartilage or bone can be irreversible. Surgical options include ankle fusion (arthrodesis) or replacement (arthroplasty). Although there is still some dispute on ankle replacements in people with haemophilia, the third generation replacement joints are better than the previous types. Range of motion and mobility regained is moderate to fair, but there is no range of motion following fusion. Llnàs believes that most people with haemophilia prefer fusions.

The third problem involves the bones and cartilage in sub-talar region (3), below the talus bone. The ankle is 3-dimensional joint and so it can be quite difficult to diagnose exactly where the problem lies. Llnàs recommends imaging with a CT scan or MRI rather than an X-ray for proper diagnosis and therapeutic planning. The bones in this area can also be fused, a procedure that can provide a lot of pain relief.



## Musculo-Skeletal Imaging Modalities in Haemophilia

*By Mike Carnahan*

New joint imaging techniques hold the possibility of earlier, more effective detection of changes in haemophilic joints according to Dr. Andrea Doria, of the Department of Medical Imaging, University Toronto and the Hospital for Sick Children, Canada. "Although X-rays remain the most widely used diagnostic imaging tool for assessment of haemophilic arthropathy, both magnetic resonance (MRI) and ultrasound imaging provide appealing advantages over radiography in detecting early changes in haemophilic joints," Dr. Doria said.

Modalities such as CT and nuclear scanning do no serve haemophilia well. The focus for haemophilia has been to detect early signs of degeneration in cartilage of weight-bearing joints. MRI is particularly good in defining soft tissue changes – such as blood deposits or residual debris from large tissue bleeds. MRI is also good for analysing changes to cartilage, any narrowing of space between joints, showing cysts and boney irregularities or changes to surfaces. There is real value in monitoring changes over time. MRI delivers critical information on sub-clinical bleeds that would not be available through conventional radiography or clinical examination.

Ultrasound is more dependent on the skill and experience of the operator and in interpretation of results.

These two imaging modalities should become more widely used in day-by-day clinical practice at haemophilia clinics around the world.

Anatomic and functional imaging techniques are at least a decade away from clinical use, but may gradually point the way toward an individualized diagnostic approach for joint care in haemophilia. Dr. Doria listed several new techniques on the horizon, including blood oxygen level-dependent MRI, ultra small super para-magnetic iron-oxide contrast-enhanced MRI and ultrasound biomicroscopy, and positron emission tomography. She said her research team at the Hospital for Sick Children has worked with international partners to develop, validate, and disseminate non-invasive measurement tools and imaging techniques. The more distant use will lie in molecular analysis.

## Sexual health and bleeding disorders

By Grant Hook

This was a new topic at the Congress and highlighted issues affecting our community. PWH are at least as sexually active as other people in their age groups and deserve to have normal lives and be sexually healthy. Sexuality and sexual health contribute greatly to overall wellbeing and need to be part of comprehensive care.

PWH are just as susceptible to sexually transmitted infections as the general population so normal age appropriate education, prevention and treatment applies.

Prevention is the best option against sexually transmitted and anything abnormal needs to be checked. Some STIs can also cause abnormal bleeding, especially in women; so don't just blame the bleeding disorder and have it looked into.

Circumcision is the most common operation in the world. Due to the risks of bleeding it is preferable to carry out the operation at 6-20 months or after 7 years. The operation should be done by an experienced surgeon with a normal treatment regime arranged with the haematologist and can be done in conjunction with other surgery. If it is requested then it should still be done as it is important for PWH to feel accepted in their own culture and communities.

Eleven percent of young PWH have iliopsoas hip bleeding episodes. In 15-18 year old males with growth spurts and sedentary lifestyles this muscle may be tight and this can cause problems in periods of high use such as sports and sexual activity. If you do experience a bleed in this area talk honestly to your health professional about the cause and treatment. If no connection has been made between sex and the injury the behaviour will continue and the bleed may not be treated correctly.

Prophylaxis should be tailored to high times of physical activity. If a PWH has a sedentary lifestyle during the day followed by a period of high activity later in the day/ evening then perhaps treating in the morning is not the best time?

Issues related to a chronic condition that affect sexuality will have a greater impact socially and mentally and may cause the person to become withdrawn.

It can be difficult talking about sex, but nurses and social workers and PWH need to get comfortable talking about it. Unless we recognize the barriers to evaluating sexual health it will be difficult to assist in improving it. Some HTCs talk about it at their annual reviews or after certain bleeds.

In summary, sex is more than intercourse. It involves relationships and emotional wellbeing. Sex can be relaxing, helps you cope with emotions, improves your self-esteem and your quality of life. It causes both emotional and muscle relaxation. Good communication with partners and health care workers is important. A little pre-planning in terms of pre-use of painkillers and researching suitable positions can go a long way.

## Meet the Real Experts - Life Stages

*By Eugene Larkin*

This session was an opportunity to hear from five men with haemophila from various countries.

Chaired by HFNZ's CEO Belinda Burnett, the session began with a short video featuring two of the speakers, Paul Wilton from Canada, and Vaibhav Nehru from India, which presented and contrasted their experiences of growing up with haemophilia in two very different countries.

Paul then spoke to the session attendees. His experience of growing up with haemophilia was generally good, although he had only recently been diagnosed as also having vWD disorder, as well as Factor VIII haemophilia. This had meant that many of his bleeds had been unsuccessfully treated over the years. It is constantly surprising how many twists and turns people take in getting a good diagnosis, and the resulting effective treatment. He spoke of the great benefits he had received from being involved in the Canadian Haemophilia Society, particularly the regular camps and activities which allowed him to participate safely as a young child, and to meet and make lifelong friends. Haemophilia was clearly an important part of his life, but not the defining aspect.

Vaibhav from India also had a very positive story to tell, despite having a lot of restrictions placed on him growing up due to the lack of factor replacement therapy available. His parents had been focussed on ensuring that he achieved well at school and obtained a good education, with the result that he had graduated as an accountant and was looking forward to starting his career.

We then heard from two men in their late 30s. One, Gabriele Callizani, was from Italy, and was a doctor himself. His presentation was based on more medical aspects, rather than his personal experience, although in the question time at the end of session he made an enlightening statement. He said that at school he didn't talk about his haemophilia with his friends. Later on in life, however, when he had connected with his national foundation and met other men who also had haemophilia, he was able to view his condition differently, and this had resulted in him taking better care of himself and consequently improving his overall health.

The other man, Yuri Zhulyov, was from Russia. The lack of factor replacement therapy available to him growing up had impacted in a large way on the health of his joints. He had made the effort to be involved in his local haemophilia society, and spoke of the importance of being committed to the community infrastructure to improve the outcome for both yourself and for those less able to advocate for themselves. Russia's vast geography and economic difficulties has meant that outcomes for people with haemophilia have been very poor for many years. His hope was that things would continue to improve with the help of the World Federation.

Finally we heard the fascinating story of Gordon Clarke, truly an elder statesman in the global haemophilia community. He was born in Northern Ireland nearly 60 years ago, and was diagnosed with haemophilia when he was around 2 years old. At about the age of seven years, he was placed in a Convalescent Home for children, and did not return home to his only family until two years later. His schooling was greatly affected by his bleeding episodes, but as he told it, upon meeting and marrying the right woman, he decided in his early twenties, to complete his schooling, which included a postgraduate degree. Eventually he managed the very hospital where he had spent so much time as a child. He has recently been made an MBE for his service to the haemophilia community, and is a wonderful example of how someone can succeed both because of and in spite of having haemophilia.





# Women & Bleeding Disorders / von Willebrand Disorder

## Women and Bleeding Disorders

*By Lynne Campbell*

Andra H James, of the Division of Maternal-Foetal Medicine in Durham, M.C, USA gave this plenary lecture.

Women who are carriers of the haemophilia gene are equally as likely as men to have bleeding disorders, although they are rarely affected by severe haemophilia. Some women have a more active expression of the symptoms of haemophilia than others. This is thought to be related to the domination of the lyonised X chromosome containing the haemophilia gene, although the exact mechanism of the dominance resulting from extreme lyonisation is not understood. *(See box for explanation of lyonisation)*

Menorrhagia (prolonged heavy menstrual bleeding) is the most common symptom experienced by "symptomatic" carrier women and it is the condition most likely to lead to diagnosis of lower levels of clotting factor in women.

Women with bleeding disorders including von Willebrands Disorder (vWD) tend to experience a range of other reproductive tract bleeding disorders. These include an increased risk of hemorrhagic ovarian cyst formation and possibly endometriosis.

Carriers with low levels of clotting factor have a higher risk of bleeding in surgery than non carriers. This necessitates the careful medical management of potential high blood loss procedures such as dental extraction, tonsillectomy and appendectomy.

Preconception counselling and diagnostic testing should be offered to women who are carriers, or who are suspected of having an inherited bleeding disorder and their pregnancy should be carefully managed. There appears to be no real difference between symptomatic bleeding complications of women carrying the gene for mild or severe haemophilia.

During pregnancy it is helpful to know if the baby is a boy or a girl. If the baby is a boy, then the pregnancy and delivery will need to be managed as if the baby is affected by haemophilia (incorporating such practises as no forceps, elective caesarean, induced rather than prolonged labour etc). Although blood clotting factor levels increase during pregnancy, post partum haemorrhage is a serious risk factor for women with low levels of clotting factor. This is often a delayed response in the mother two to three weeks following the baby's birth. Post partum haemorrhage is not a high risk factor for women with vWD..

If the baby is a girl, it is useful to know if her clotting factor levels are low at an early age in order for her mother to teach her daughter in the self management of her symptomatic condition. In most countries a girl cannot undergo full genetic testing for haemophilia until after puberty and she has reached the age of informed consent.

Obstetric and gynaecological problems can affect all women. There is overwhelming evidence to show that women with bleeding disorders are far more likely than the average female population to experience conditions that manifest with bleeding beyond their child bearing age. These include fibroids, endometrial hyperplasia and polyps. Quality of life is a major factor for all women with bleeding disorders and for this reason a woman with a bleeding disorder is advised to seek optimal management of her bleeding disorder.

To undergo hysterectomy and to have a hysterectomy at an early age has been a drastic choice and consequence for many women with low levels of blood clotting factor. With careful management such radical intervention can be avoided. Tranexamic acid, the intrauterine device Mirena and the contraceptive pill have all proven to be successful interventions in reducing bleeding for women with inherited bleeding disorders.

## Carriers

*By Chantal Lauzon*

In an interesting session, speakers from different parts of the world examined issues related to being a carrier of the haemophilia gene. Although many of the speakers were not fond of the term 'carriers', they agreed no better term was currently being used. There are many aspects to being a woman who carries the haemophilia gene (carriers tests, psychological impact, bleeding symptoms, reproductive choices, prenatal diagnosis, having a child with haemophilia, medical and social issues) and each of the speakers concentrated on a different aspect.

Richa Mohan (India) discussed the impact of diagnosis

### Lyonisation

Women have two X chromosomes, and in any given cell one chromosome will be active (designated as Xa) and one will be inactive (Xi). X-inactivation occurs so that the female, with two X chromosomes, does not have twice as many X chromosome gene products as the male, which only possess a single copy of the X chromosome. Which X is inactivated is random process, but if more of the chromosomes with the haemophilia gene remain activated (so the haemophilia gene is on the Xa) then the woman will usually have lower factor levels and will experience more bleeding symptoms.

of carrying the haemophilia gene in the Indian culture. Social stigmatisation is a major issue and creates a lot of anxiety for women who know haemophilia runs in their families. This can have an impact on marriage, either after a child with haemophilia is born, or when it comes to pre-arranged marriages. It was considered better to inform the other families before a pre-arranged marriage in the case of obligate carriers as the consequences of knowingly hiding this information would be worse if a child with haemophilia is born from the union. Within families, the subject of haemophilia is often not talked about and girls are not always told of the risk of carrying the condition. This being said, emotional support from families was considered essential in times of crisis. Mohan has helped set up a counselling service for carriers in her area with support groups. There is a high interest in carrier testing and prenatal diagnosis. The birth of the first son is very important to a woman's status in the family so the concept of abortion can be problematic. Often husbands are not very involved with raising children or their medical care, so the burden rests on the mother.

Anne Gillham from South Africa painted a different picture of the issues of being a carrier in her country. She spoke mostly of their centre's experience of factors that have influenced the uptake of genetic counselling. Anonymous genetic counselling is available to all people with haemophilia at their clinic, however, very few people were using the service. They conducted personal interviews with a number of obligate carriers, suspected carriers and mothers of children with haemophilia about their awareness of the service and thoughts on it. Those that had undergone genetic counselling were all mothers of children with haemophilia and had found the service helpful. Those that had not undergone counselling had poor understanding of the risks of passing on the condition, many had not heard about it and some did not want to know about it. Not a single person interviewed had received the letter the clinic had given to people with haemophilia to pass on to their female relatives. There was a perception that the haemophilia clinic was there for the boys/men and not (potential) carriers. The issue of non-disclosure in families was also an issue. The condition was sometimes not explained by mothers until a grandson was born, even if a woman's father had haemophilia. In terms of impact on family life, the situation was different to India in that husbands were perceived to be very supportive. Recommendations from the study were that it was important to educate patients about haemophilia so they can properly communicate it to their families and to set up a clinic day specifically for women with bleeding disorders and carriers.

Clare McClintock, an Obstetric Physician and Haemotologist from Auckland City Hospital spoke about women and the haemophilia gene. She pointed out that in fact that men also 'carry' the haemophilia gene, not just woman. Calculations have been done and for every 100 men with haemophilia there are 277 women that need counselling and testing for the haemophilia gene, of which 156 will be carriers. According to WFH figures, there are 750,000 female haemophilia carriers in the world.

McClintock explained that some women who carry the haemophilia gene are more symptomatic than others due to gene lyonisation *(see box for more an explanation)*. If more of the X chromosomes inactivated are the 'healthy' Xs without the haemophilia gene, then the woman will usually have lower factor levels and will experience more bleeding symptoms.

Carriers usually had lower clotting factor levels than non-carriers and usually experiences more bleeding after operations and dental extractions and required more treatment for bleeding. Few carrier women experienced spontaneous bleeding, but the lower the clotting levels the higher the severe the bleeding, especially menstrual bleeding.

In pregnancy, factor VIII levels rise but not factor IX levels. Woman should be closely monitored for post-partum bleeding and have a delivery plan in place if having a boy who might have haemophilia. Intracranial haemorrhages in the newborn are to be avoided at all costs, so ventuse deliveries should avoided and forceps and suction should not be used unless prenatal diagnosis has determined the baby does not have haemophilia. There are high rates of caesarean sections in carriers in the UK and the USA, but there is no clear evidence that caesareans are better than vaginal deliveries if are pre-planned. Emergencies caesareans come with the similar bleeding risks as difficult vaginal deliveries for the baby. Caesareans do increase the risk of post-partum bleeding in the carrier woman.

Top tips for woman who carry the haemophilia gene:

- Remember that you are probably not the only person in your family that can pass on the gene
- Try not to focus on the bad gene – you've got thousands of good ones
- Pay attention to bleeding symptoms
- Treatments are available, seek out a good doctor
- Keep a sense of humour

Finally, Ulf Tedgard (Sweden) spoke on the management of bleeding in women. He noted many carriers fit the definition for having mild haemophilia. He thinks factor levels should be measured at a young age if there are signs of a tendency to bleed or before surgery, but that genetic carrier testing for a definite diagnosis is best done after puberty or when the young girl can give informed consent for the test.

Management of bleeding in women who carry the haemophilia gene requires a multidisciplinary approach. It depends on factor levels and haemophilia type, whether factor concentrates are needed or whether products like desmopressin and tranexamic acid can be used. Tedgard suggested that factor levels should be tested before each surgical procedure so that a treatment protocol can be put in place.

Menorrhagia, or heavy menstrual bleeding, is a common problem in women with inherited bleeding disorders, especially in those with vWD. Iron deficiency from anaemia is also common. Menorrhagia can have a heavy toll on quality of life as it limits physical activity, is accompanied by pain and also affects mental health, vitality and social functioning. A gynaecologist should be involved in the management as menorrhagia could also be due to other causes then low factor levels and these should be investigated properly. First line treatment is tranexamic acid and, for women who are not trying to conceive, the combined contraceptive pill or Mirena. Desmopressin can also be used in those with low factor VIII levels or vWD. For those who do not wish to preserve fertility (already had children, etc), surgical options include endometrial ablation and hysterectomy. Tedgard cautioned to avoid NSAIDs (aspirin, ibuprofen, etc) as pain relievers and make sure to treat the iron deficiency.

If a carrier is planning on having children, factor levels should be checked before pregnancy and at 32-34 weeks or prior to any invasive procedures. If factor levels are below 50%, he suggests giving clotting factor during delivery with a recombinant product (to avoid possible risk of parvovirus B19 infection from plasma-derived products). Desmopressin can have a serious effect on water levels so maybe not the best choice for use during delivery. Tranexamic acid can be used if factor levels are borderline. He recommends trying to keep factor levels above 50% for at least 3 days after delivery to avoid serious post-partum bleeding.

In summary, there are few reliable studies in bleeding problems among carriers of haemophilia. It can have a serious affect on quality of life but adequate treatment is available. HTCs should take greater interest in carriers of haemophilia and their problems.

## Role of Genetic Diagnosis in vWD

***By Chantal Lauzon***

Paula James (Canada) explained how the von Willebrand Factor (vWF) molecule is subject to many genetic mutations. As such, genetic analysis can be difficult, and diagnosis usually relies on family history, symptoms and lab testing.

Type 1 is the most challenging to diagnose genetically. Levels of vWF can change with time and due to stress and other factors. It is therefore important to have repeat lab testing for vWF levels in suspected cases of vWD. Genetic studies have shown that that not all people diagnosed with vWF had a genetic mutation in the vWF gene (59-70%). The lower the level of vWF, however, the higher the chance is of finding a mutation. There are lots of different types of mutations found, but genetically there are really two groups of Type 1 vWD: highly penetrant and incomplete penetrant. The highly penetrant group has a clear mutation to the vWF gene. The incomplete penetrant group may have low levels of vWF, blood group O but no vWF mutation can be found (around 30% of cases). James concluded that it was too early to do genetic testing for clinical diagnosis of Type 1 vWD.

For Type 2 vWD, however, genetic testing can be helpful for clinical diagnosis. There are three well known gene mutations responsible for Type 2a and one for Type 2b. Type 2b is characterised by thrombocytopenia (reduced platelet count) that is usually made worse by stress. Two specific genetic mutations that affect binding of vWF to collagen are responsible for Type 2m. People with Type 2m usually respond poorly to desmopressin and clotting factor replacement is needed for treatment. Type 2n can be difficult to distinguish from haemophilia A, as factor VIII levels are often lower than vWF levels. Genetic testing can, however, differentiate between the two, which can be critical for proper treatment.

Type 3 vWD is characterised by an absence of vWF and low factor VIII levels. Type 3 vWD is inherited in an autosomal recessive pattern. Parents each carry a gene responsible for a milder form of vWD and can experience a range of symptoms, from none to many bleeding problems (nose bleeds, heavy periods, etc). The child with Type 3 has inherited both vWD genes and has severe bleeding symptoms, similar to those seen in severe haemophilia. Of published genetic studies of people with Type 3 vWD, there are lots of locations for mutations throughout the gene. Further research studies are needed to truly be able to use genetic testing for diagnosis, however, in the mutation in both parents is known it would be possible to do prenatal testing.

Overall, when applied rationally, genetic testing can be a helpful addition to other laboratory investigations in vWD.

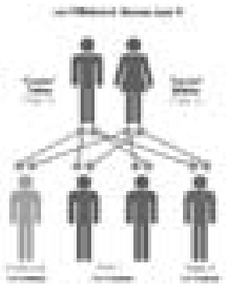


**Figure 6.**
Inheritance pattern of von Willebrand Disorder Type 3.




2010 WFH Hemophilia World Congress

# Rare Bleeding Disorders

## Living with a Rare Bleeding Disorder

*By Lorraine Bishop-Porter*

After Latifa Lamhene (Algeria) was diagnosed with factor VIII and factor V deficiency, she set upon a massive search to understand her condition. Having several serious bleeding episodes and numerous operations spurred her into action to spread the world for people to have a better understanding of the disorders. Latifa said her objectives are to fight the ignorance and loneliness caused by bleeding disorders. She eventually became the president of the Algerian Haemophilia Association.

Evelyn Grimberg (The Netherlands) discussed living with Glanzmanns thrombasthenia. She explained this disorder, how it is inherited and types of treatments available. She felt her life was more positive now that she has a better understanding and has made contact with other people with the same disorder. Just recently she had attended a Dutch haemophilia society youth camp for people with bleeding disorders.

Gabriel Lottas (Switzerland) has fibrinogenemia or factor I deficiency. Gabriel gave a very interesting account of his life and his personal experience. When a baby of three months he suffered a gastric perforation and was lucky to survive a laparoscopy. He also recounted the day he decided to follow in his father's footsteps and shave with a straight razor, instead cutting his lips (Kids please don't try this one). Gabriel missed many school days due to bleeding episodes, however, once he started a prophylaxis treatment things improved. Mastering the ability to self-treat has enabled him to travel and gave him more freedom.

## Platelet Disorders

*By Chantal Lauzon*

Sarah Israels of Canada spoke about how the many platelets abnormalities can be divided into five main groups depending on how they function. For example in Bernard Soulier Syndrome, the platelets fail to integrate or adhere to the blood vessels because a receptor on the surface of the platelets needed for binding to vWF is missing.

Magdy El-Ekiaby of Egypt described Glanzmans Thrombasthenia which is also caused by a defect in platelet membrane receptors. A rare disease (1 in a million), it is an autosomal recessive condition that leads to a moderate to severe bleeding disorder. With symptoms such as menorrhagia, bruising, nose and mouth bleeds and gastrointestinal bleedings, it is important to diagnosis the condition properly and eliminate other conditions such as vWD and fibrinogen defects. Diagnosis includes reviewing the symptoms, doing laboratory tests and taking a careful family history as the condition is more common in communities where marrying cousins or other relatives is common. For minor bleeding, treatment is conservative and products like fibrin glue can be used. In the case of major bleeding, platelet transfusions are often needed. There has also been some experimental use of rFVIIa.

Finally, Teresa Quiroga spoke about bleeding of unknown cause (BUC) and the difficulties in diagnosing rare bleeding and platelet disorders. Symptoms can be mild to moderate, but can have a big impact on patients and a present a big diagnostic and treatment problem for clinicians. One of the problems is it can be quite difficult to differentiate 'normal' bleeding from 'abnormal'. There is a high prevalence of people with known bleeding disorders in the population (vWD, platelet function), high prevalence of bleeding symptoms in the 'normal' population' and many people with genuine bleeding problems with no known diagnosis. The symptoms are often the same and are sometimes related to another health problem, not a bleeding disorder. Diagnosis is also made difficult because most screening tests are non-specific and some patients with a known inherited bleeding disorders have 'normal' blood tests, so further investigation and families histories are very important.





# Hepatitis C and other Viral Pathogens

## Viral Pathogens

*By Chantal Lauzon*

Parvovirus B-19 continues to confound manufacturers' efforts to eliminate it from both recombinant and plasma-derived products, although several promising developments have been reported. Dr Jeanne Ann Jordan explained that parvovirus B-19 is an extremely small, non-enveloped virus, barely half the size of HCV. As a result, it is highly resistant to solvent and detergent treatments, heat inactivation, and fractionation.

More than half of those infected with parvovirus B-19 are completely asymptomatic, and it leads to a mild, self-limited illness in most others, with symptoms such rash ('slapped cheek'), joint pain and fever. In high-risk populations who are immune-compromised, however, the symptoms can be much more severe. They include severe anaemia, and sharp drops in red and white blood cells and plasma. In pregnant women, infection can be passed to the developing foetus and result in spontaneous abortion. There are almost no treatment options available except for the use of blood transfusions to treat the symptoms.

Jordan explained that one of the major challenges in trying to ensure the safety of the blood supply is that there is a long period without symptoms and an early period of rapid rise of virus levels in the bloodstream. Transmission most often occurs when a person donates blood during this window.

Recent studies estimate that the prevalence of parvovirus B-19 among blood donors varies from one in 531 in Japan to one in 5,950 in France, Jordan said. Products made from pooled donations and clotting factor concentrates appear to have the highest rates of infection.

There is reassuring news on the horizon, however. Nanofiltration has been used to eliminate small, non-enveloped viruses like B-19, and NAT testing is allowing the removal of material with the highest viral load to reduce risk. In addition, Jordan said manufacturers are developing nanoexchange and chromatographic techniques to eliminate the virus.

HIV and hepatitis C (HCV) both continue to be viral pathogens of significant concern to people living with bleeding disorders. Dr Margaret Ragni noted that HCV infection is the leading cause of end-stage liver disease in people with haemophilia. Other high-risk factors of end-stage liver disease include hepatitis B infection (past or present), alcohol use, age and length of HCV infection. In those with HIV co-infection, she said research indicates that treatment with highly active anti-retroviral treatment (HAART) also improves HCV symptoms.

In people with haemophilia and HCV, Ragni recommends:

1. early referral to a hepatologist;
2. monitoring for fibrosis with blood markers of liver function such as alpha-fetoprotein;
3. monitoring for end-stage liver disease using the MELD score (tool available online); and
4. using antiviral therapy if also co-infected with HIV.

In the USA, they regularly do liver biopsies on patients with haemophilia and HCV and maintain this is the best way to test for liver damage. Other experts in the room did not agree with the use of liver biopsies in people with bleeding disorders.

Dr Kenneth Sherman explained how many different agents are being developed to treat HCV. Every point of the HCV life cycle is a chance to stop the virus multiplying. Some block the entry of the virus into a cell, some block how the virus reads it RNA and some block enzyme functions needed for replication. This last group, the Direct Acting Antivirals (DAAs) which includes protease inhibitors, has been the most studied. Two protease inhibitors, telaprevir and virceptravir, are both in phase III trials. Before these drugs can be approved for patients, the FDA has stated that they need to show superiority to current treatment (not just equivalent outcomes). After various combinations, it seems that they work best when combined with current recommended treatment and lead to high sustained viral response ("cure") rates.

One trial in New Zealand (INFORM trial), tested two DAAs on their own (one polymerase and one protease). The results looked good and no resistance developed, but because patients went on to standard treatment after 12 weeks of the DAAs alone it is hard to draw clear conclusion from the trial.

HCV is one of few viruses where there is a real cure. Sherman said there are many promising new drugs in development for the treatment of HCV, but cautioned that pegylated interferon would continue to be a part of active treatment in the foreseeable future, as single-drug treatment tends to be unsuccessful because of the high levels of mutation of HCV and its ability to adapt very quickly to new environments. The new DAAs have almost all been targeted to harder to treat HCV genotype 1, and pegylated interferon plus ribavirin will continue to be best practice for patients with genotypes 2 and 3.

## Psychological aspects of HIV and HCV infection

*By Mike Carnahan*

Anthony Roberts, of the South African Haemophilia Foundation, reflected on psychological aspects of human immunodeficiency virus (HIV) and hepatitis C virus (HCV) infection. Living with potentially ominous viral infections such as HIV or HCV can engender a range of psychological effects. Some of the most commonly reported reactions are fear, anger, anxiety, sadness, depression, and lowered self-esteem. When one adds haemophilia to the above scenario,

the situation becomes one of "multiple woundedness" and can lead to suicidal thoughts or withdrawal from social activities. An inventory of 'woundedness' can help to unpack the hierarchy of 'woundedness' as well as the effects of the various emotional responses.

Alternately, living with haemophilia can also be a gift of awakening a deep sensitivity and a strengthened approach to life. This can lead to a different range of psychological effects, some of which are hope, surrender, acceptance, peacefulness, and spiritual enlightenment. The decision of where to position oneself amidst all these emotional responses is a complex exercise. It is, most often, a combination of despair and hope, where periods of restfulness are followed by periods of hopelessness. The influences of role-modelling, culture, and economic status were elucidated.

## Meet the Experts – Hepatitis C

*By Lynne Campbell*

Chair: Jorge Daruich, Argentina

Panel: Kenneth Sherman, MD, USA and Elizabeth Paradis, RN, Canada

Hepatitis C is a major problem for those with haemophilia. In this session, the Chair employed the unusual strategy of posing a series of rapid fire questions to the two panel members himself. Most questions were quite direct and most specifically addressed medical issues and were answered by the M.D.

Topics of interest included:

- **How patients could avoid transmission of the virus in their daily lives:**
  - Essentially this is done by avoiding blood exposure. If people cut themselves then diluted bleach should be used to clean contaminated surfaces
  - Toothbrushes and razors should be stored away from those used by other family members
  - Those with HCV ideally should not share ear rings or carry out belly button and nose piercings using needles at home
  - Sexual transmission to spouse: HCV is difficult to transmit but 25% of transmission is via sexual contact. Transmission risk is higher early in a relationship. Condoms are not necessary for long term monogamous pairs but are necessary for others. When a woman with HCV has menorrhagia, then a condom should be used.
  - Tattooing is a risk factor as the same ink tends to be used even if the needles are sterilised. In reality often first tattoos are not done hygienically or professional, so HCV contamination is a risk.
- **Food Hygiene:**

On diagnosis it is important for the person with HCV to also receive the Hepatitis A vaccine to protect their long term liver health.

  - Observe good food hygiene practices while preparing food.
  - Although no food product will make the liver better, poorly prepared food in some overseas destinations is a risk factor for the person with HCV.
  - Alcohol is detrimental to those with liver disease and should not be taken.

Vitamins and supplements should be avoided as many "natural" products are toxic and damaging to the liver. Retinoids, carotenes and fish oils stimulate collagen and risk scarring to the liver.

- **Diagnosis of Hepatitis C:**

The test for HCV has high sensitivity and low specificity. Low levels of HCV can exist in the blood and not be detected. Repeated RNA testing is required, so if a person's HCV RNA reading is negative, two more negative results are required before the patient can be confident the virus is cleared.

- **Liver Biopsy:**

A person with haemophilia can be put onto treatment without a liver biopsy.

Kenneth Sherman believed the liver biopsy is the most informative type of diagnostic tool available for assessing the health of the liver. Those with genotype 2 or 3 HCV disease respond well to treatment; however, for those with genotype 1 and 4, not only does he believe it is more beneficial to undergo a biopsy, but he is adamant that this can be done safely in those with haemophilia. He believes ultrasound is of no value in determining the extent of disease. Fibroscan measures liver stiffness and provides an answer, but he considers the answer is wrong 30 - 40% of the time, so he does not consider Fibroscan appropriate or useful. Kenneth Sherman's preference for diagnostic purposes is trans- jugular liver biopsy, provided a large enough sample can be procured.

The Canadian nurse did not agree and commented that liver biopsies were not performed in people with haemophilia in her practice.

- **Importance of studying insulin resistance:**

Insulin resistance is associated with diabetes. Diabetic patients have a lower response to HCV treatment. A liver biopsy can determine if the fatty liver is alcohol related. Patients with type 2 diabetes should first lose weight (10 lb recommended) then start vitamin E supplementation which is safe for liver health.

- **When would you not treat?**
  - If an evaluation of the HCV patient's mental state detected psychological instability then treatment would not be stated as the patient would be less likely to carry through with treatment.
  - If a patient was healthy and had not received treatment for 20 years, Kenneth would tell his patients that better treatments are coming and treatment could be delayed.
  - If other health concerns, e.g., HIV, are an issue, then to achieve an undetectable viral load must come first. HIV treatment would also provide medical staff with an indication of how well a patient will comply with their HCV regime.
  - Other liver diseases would need to be considered first.
  - Psychiatric and drug dependency issues would also need to be addressed first.

- **Treatment – When do you start and when do you wait?**

Warehousing is the term given to waiting to treat in anticipation of a new and better treatment option. New drug development is targeted at those with genotype 1 HCV, therefore warehousing is not utilised for those with type 2 or 3 HCV.

When patients have stage 3 or 4 compensated liver impairment then it is better to treat now.

- **When would you recommend a short course of treatment or long treatment?**

The side effects of HCV treatment are significant and can impact on the time a person remains in treatment. For genotype 2 or 3 treatment time is 24 weeks, for genotype 1 and 4 the duration is 48 weeks. For those with a co-infection then 48 weeks treatment is required.

Patients who have a rapid viral response exhibit a very good response rate with shorter periods of treatment. Longer treatment periods (48 to 72 weeks) tend to have a high drop-out rate because of drug fatigue. Although genotype 2 and 3 patients can be treated in a short time of 12-16 weeks, studies show that those who undergo therapy for 24 weeks did better overall.

- **Side effects:**

HCV treatment can cause nausea, sleeplessness, flu like symptoms and low red blood cell count leading to fatigue. By six months depression sets in. These symptoms all need to be managed.

Men typically get irritated and angry, women get sad and withdrawn.

A good nurse co-ordinator is essential to relate to the patient, assess mood and to arrange medications to address specific side effects.

- **When to stop treatment:**

Essentially treatment should not be stopped too early. Better outcomes are achieved when the highest possible dose is maintained to beyond week 20.

At 24 weeks patients ideally achieve zero viral load. Re-test after a further six months, if the viral load is still zero then the patient is deemed to have cleared the virus and is cured.

If the patient already has fibrosis, even if the virus has gone, the patient will require ongoing screening.

- **If a patient is a non responder, what do you do?**

First, you need to determine what type of non responder they are. The implications and decisions for each type of non responder are different. If a person with HCV had been inadequately treated previously or treatment was stopped, then the patient would be encouraged to undergo re-treatment.

- **Finally - What are the known symptoms of the new treatments?**

New therapies result in different side effects as each new drug has new characteristics. Increased anaemia and rash are still a problem with the new drug regimes.



## Additional comments on Hepatitis C

*By Mike Carnahan*

Dr Kenneth Sherman of USA said in the US 25% of patients with HCV have stage 3 fibrosis. It is now possible to make predictions for individual patients about their HCV prognosis. A scoring system applying indices to factors that will contribute to liver fibrosis –the presence of active HBV, consumption of alcohol, number of years with the disease, blood test results, whether HCV treatment has been embarked on and age.

Dr Sherman gave the view that pegylated interferon would be the standard drug for the foreseeable future but 138 drugs are presently in trials. The most promising is a protease inhibitor - Telaprevir. Trials are showing 50% of previously treated genotype 1 patients are now achieving a sustained response to treatment with telaprevir combined with standard therapy.

Brian O'Mahony of the Irish Haemophilia Society, Dublin, described the current status of worldwide compensation for HCV. Compensation for persons with haemophilia with HIV or hepatitis C has been a defining issue for many haemophilia societies over the past 20 years. WFH is aware of eighteen countries where compensation for HIV infection has been paid. Payments were made by the government in all eighteen of these countries, with pharmaceutical or insurance companies also contributing in ten of the eighteen. Hepatitis C compensation has been paid in ten countries. The government has paid compensation in nine countries with the insurance company paying in one country. Several countries continue to campaign for hepatitis C compensation.

Haemophilia societies must carefully consider embarking on or continuing a compensation campaign. Clear objectives must be agreed by the board and by the members, including those not infected by hepatitis C. Financial objectives should be realistic, practical, and achievable, and the current economic climate at any given time must be a consideration. Compensation does not necessarily bring closure and other priorities of the organisation may be adversely affected. A compensation campaign, if not properly planned and executed, can last for several years and can drain the resources, enthusiasm, and motivation of the organisation, staff, and volunteers.

## Late Breaking Medical Topics

*By Chantal Lauzon*

Although there are still no confirmed symptomatic cases of **variant Creutzfeld-Jacob Disease** (vCJD) transmitted through haemophilia treatment products, a recent UK study has confirmed a likely case of asymptomatic transmission to a person with haemophilia, according to Prof. James Ironside. Last year prions, the causal agent of vCJD, were found in the spleen of a 70-year old person with haemophilia in the UK on autopsy. The person has never had symptoms of vCJD and had died of unrelated causes. After careful examination and risk analysis, it was concluded that the prions were most likely from unimplicated low-purity UK plasma-derived FVIII products, rather than by chance, from blood transfusion or batches of FVIII known to contain plasma from a donor who later developed vCJD (donor had a different genotype of vCJD as those found in the spleen).

Although there is currently no commercial test for vCJD for people at risk or in blood donations, Ironside said that if the levels of infection were truly very high in treatment products then they'd have seen many more haemophilia patients with active vCJD in the UK. All people with haemophilia in the UK treated with plasma factor concentrates between 1980 and 2001 have been informed of the risk and have been invited to participate in a study of tissue-based evidence of vCJD infection in the event of surgery or after their death.

Dr Victor Blanchette of Canada then gave an **update of recent clinical trials**.

The Canadian Hemophilia Primary Prophylaxis Study (CHPS) began in 1997 and has followed 56 boys with haemophilia on prophylaxis with recombinant FVIII (funded by Bayer). It is a dose-escalation study, so the boys started once-weekly prophylaxis after their first bleed, then moved on to 2x/week and then alternate days if they continued to have bleeds. There was a specific 3-day treatment in the case of break-through bleeding. As the boys got older they almost all moved on to alternate day treatments but each at a different rate. Follow-up has shown no clinical symptoms of joint damage, but MRIs have revealed some mild joint damage in some boys' ankles (loss of cartilage)by the age of 8 years. The significance of the findings are unclear at present and the study will continue to follow-up the boys.

The European Initiative to Prevent Damage in Haemophilia A Children with Inhibitors (ENJOIH) is a study that is about to get underway. They are looking to recruit 50 patients from European countries to evaluate the efficacy of prophylaxis with rFVIIa in boys with haemophilia A and inhibitors in reducing joint bleeds. Participants will be randomised to on-demand treatment or prophylaxis.

The SIPPET study is looking at the development of inhibitors in patients on plasma-derived treatment versus recombinant treatment. Investigators plan to enrol 300 patients in many countries around the world, although not in those that use recombinants as first-choice in children like Australia and New Zealand.

The RESIST studies concentrate on immune tolerance (ITI) for patients with inhibitors. There are two different subsets to the study, patients who have never tried ITI and those who have but still have inhibitors. Participants will be enrolled on an ITI course with a plasma-derived FVIII concentrate that also contains von Willebrand factor (vWF), the idea being that the presence of vWF may help with the success of ITI.

The challenge common to clinical trials in bleeding disorders are designing a study to clearly show study end-points, recruiting enough patients for the results to be significant, and getting appropriate funding support for the product needed to do the trials. International collaboration is needed because there are many unanswered questions in haemophilia care.

Dr Donna DiMichele from the USA also reported the preliminary results from the recently terminated International Prospective Randomized Immune Tolerance Study which has revealed some clinically differences between high-and low-dose regimens for inducing immune tolerance. The trial was stopped in November 2009 both for futility and safety reasons, so the data presented was close to final. The study looked at the role of dose in ITI of haemophilia A patients with inhibitors (200IU/kg/day vs. 50 IU/kg/day) and followed up patients while on ITI and for an extra year to check for relapse. The average age of patients was 15 months and 78 finished treatment before the trial was stopped, with around 70% achieving immune tolerance. There was no statistical difference between the high-dose and the low-dose arms in achieving ITI success, but the time it took to achieve success was quicker in the patients receiving the high dose ITI. Once tolerance was achieved response was similar however. One of the reasons the trial was stopped is that it would have taken over 400 patients to show complete equivalence between the two arms and only 134 had been recruited so far. Product used was the choice of the treating clinician so some were on plasma-derived and some on recombinant.

In terms of safety, most adverse events were catheter/port related. Catheter infections did not appear to have a difference on ITI outcomes. There was more bleeding in the low-dose arm at the beginning before tolerance was achieved.

The clinical practice lessons are that the daily regimen seem to have advantages, but which daily dose to use is not yet truly known. The high-dose used in study protocol is twice what is commonly used in the USA for ITI (100 IU/kg/day). The cost-effective analysis might shed further light on these results to help inform clinical choices.

## Special Lectures in Haemophilia Management

*By Chantal Lauzon*

Gene therapy may soon reach the point where it can either reduce the risk of inhibitor formation or treat it after it has occurred, Dr. Katherine High (USA) presented.

"This is a very exciting time for gene therapy," she said in an interview. "There's been more of a spotlight on other diseases in the very recent past. But I think there will be very exciting things coming out about haemophilia soon, and it's reasonable to think about this approach."

Dr High said there is only limited research data available from the 43 human participants in gene therapy trials to date. The wealth of animal data shows that gene therapy does not necessarily protect against inhibitor development, and carries a variety of risk factors. However, animal studies do demonstrate that "you can induce tolerance to the factor IX protein, even in an animal that is not normally tolerant to it, and that's a pretty robust conclusion," she said.

A member of Dr. High's team, Dr. Valder Arruda, is working with canine subjects in an effort to eradicate inhibitors using gene therapy. The approach uses a gene therapy vector to for continuous delivery of factor VIII protein, so you introduce the vector into the liver and continuously produce the clotting factor.

Research is also addressing "the likelihood that you could actually eradicate inhibitors using a gene therapy approach." Results so far are promising, but the mechanism of action is not well understood, largely because the reagents for looking at the cellular basis of immunologic phenomena are not as readily available for dogs as they are for humans and mice.

The safety of any treatment would have to be established in adults before it could be considered for children, but Dr High cited one difference in biology that could bode well for a gene therapy that involves introducing a vector to the liver in children.

"There are some subtle differences that we know of from studies in animals," she said, "and one of those is that that the liver in the younger animal is still growing. Instead of a population of cells that are mostly cycling very slowly, you have cells that are still dividing, and that could increase the probability that the vector will actually integrate into the DNA."

Dr Alessandro Gringeri (Italy) discussed the immunogenicity of plasma-derived and recombinant factor VIII concentrates as a risk factor for inhibitor development that is potentially treatment-related, and is probably modifiable. From the data available, however, there doesn't appear to be a difference in the frequency of inhibitors in people using plasma-derived factor VIII concentrates compared to those using recombinants. He then outlined the protocol of the upcoming SIPPET (Survey of Inhibitors in Plasma- Product Exposed Toddlers) study aimed at revealing the true incidence of inhibitors in patients treated with either product which has recently begun enrolment.

## Novel Therapies

*By Richard Scott*

Claude Negrier (France) gave a history of factor products from human plasma to fractionated plasma, and then the first and second generation recombinant products. Then he looked at what is in store for future products. He said the evolution of recombinant technologies points toward improved quality of life and reduced treatment costs.

While third generation recombinant products are largely devoid of traces of human or animal proteins, newer products have potential advantages such as improved pharmacokinetics, higher production levels and lower costs. Fourth generation recombinants have already completed animal trials and human trials will begin soon. These molecules tend to have a longer half life and fewer traces of animal protein. Fifth generation molecules are also underdevelopment but still a way off from trails. These molecules will have longer half-lives, fewer traces of animal protein, and reduced immune reaction (i.e. less inhibitors).

Bio-engineering of molecules to provide longer half life will improve prophylaxis by changing it from three injections to perhaps one per week.

Different strategies of extending the half life have been tested:

- Companies have tried pegylation using polymers polyethylene glycol to surround the clotting factor. Pegylation is the process of attaching of polyethylene glycol polymer (like sugar) chains to another molecule, normally a drug or therapeutic protein. While this did provide a longer half life it also changed the effectiveness of the molecule.
- Pegylated liposomes were found not to be effective. The bleeding survival rate (mouse model) improved but bleeding rates remained the same.
- With the B Domain deleted FVIII molecule (i.e., Refacto) pegylating the molecule increased factor presence from 30 hours to 50 hours. Inhibitors react to certain parts of the FVlll molecule and human antibodies recognise less of the B domain deleted molecules so the factor lasts longer.

Glyco-pegylation is also being trialled with FVIIa . Glycopegylated FVIIa achieved a 15hr half-life instead of current 2.5hrs. Natural glycol added to activated FVIIa improves binding to active platelets increases circulating half life. Longer lasting factor is more likely to be applied in FVIIa first due to its current short half life. New molecules are also more effective. Adding to the molecule improved clotting.

Other developments include:

- Transgenic animals that produce FVII molecules via milk. This would lower production costs reducing the cost of factor.
- More drastic modification, such as the fusion of molecules FVIII and FVIX.

- Fucoidan Pro-coagulant could assist with oral FVII treatment. Thrombin generated from oral consumption and brown seaweed plant based polysaccharides are being tested in dog models. Cuticle bleeding time was reduced.

So there are some encouraging signs that longer life factor will be available once further testing is complete and approval granted. The cost of production and volume of factor produced could be possible via transgenic milk producing animals. In the more distant future other products may be taken orally.

## Mild Haemophilia

***By Chantal Lauzon***

Katheligjne Peerlink (Belgium) delivered a plenary speech on mild haemophilia on Tuesday morning. She spoke about how the haemophilia world is realising that people with mild haemophilia are the majority of patients and severe complications are more common than previously thought.

Mild haemophilia is usually defined as having factor levels of between 5%-30% of normal. Symptoms include bleeding for a long time after surgery or a bad injury, but then again bleeding is not often and usually not unless there is an injury. Prevalence of mild haemophilia if difficult to calculate as reports vary. According to the WFH Global Survey, 32% of people with haemophilia are mild, but the proportion is closer to 50% in countries with more complete registries such as Canada and Spain.

Diagnosis is usually made following a bleeding episode due to an accident unless there is an established family history. Diagnosis is usually made at a much older age than severe haemophilia, usually later childhood or adulthood.

Mild haemophilia A is usually caused by gene mutations that either lead to decreased production of FVIII, impaired interaction of FVIII with vWF, or reduced activity of FVIII. Laboratory diagnosis can be made difficult due to how levels of FVIII can fluctuate due to exercise, bleeding and fainting, etc. The test are designed to measure the activity of normal FVIII but with mild haemophilia the problem can sometimes be the quality not the quantity of FVIII, as some cases of mild haemophilia A will be missed depending on the test used. Activated FVIII has reduced stability in 30-40% of people with haemophilia A. Unfortunately, there is no consensus on which test is best.

Problems with the management of mild haemophilia include delayed diagnosis and delayed treatment after injury because bleeding episodes are infrequent and can go unrecognised at first. Desmopressin can be used for the treatment of bleeds in some people with mild haemophilia A, but factor concentrates are most often used. Inhibitors of fibrinolysis, such as tranexamic acid can also be used.

One large study has shown there inhibitors develop in around 5% of people with mild haemophilia A. Inhibitors can lead to a change in bleeding patterns, essentially changing the severity from mild to severe including spontaneous bleeding, or in others there is no change in bleeding pattern but FVIII replacement no longer works. Risks factors for developing inhibitors in mild haemophilia A include periods of intensive treatment with FVIII concentrates and a genetic predisposition. The body seems to be able to distinguish between the circulating self-produced FVIII and foreign FVIII. There is no specific eradication treatment for inhibitors in mild haemophilia as ITT doesn't appear to work as well. Many inhibitors in mild haemophilia seem to disappear spontaneously with time. Treatment should be individualised to the patients and could include using desmopressin or bypassing agents instead.

It is important for HTCs to have regular follow-up of patients with mild haemophilia and re-test factor levels from time-to-time. As a person with mild haemophilia ages they will encounter different health problems that might require more careful management due to their bleeding disorder (blood pressure, heart conditions, cancer, etc.).

## Gene Therapy

***By Mike Carnahan***

Dr David Lillicrap, Professor in the Dept of Pathology and Molecular Medicine, Queens University, Canada, pointed out diseases with a single cell defect are candidates for gene therapy. For haemophilia, research into gene therapy began in 1984 and already some of the results of that research into gene therapy are in use with prenatal diagnosis and recombinant products.

Despite setbacks, a large number of trials are still going on, particularly phase I and II trials. Selecting the right vector (or transporter) continues to be a major issue as the haemophilia gene is a "large cargo" for a transporter. Viral vectors continue to be the main vectors for haemophilia with transportation being directly to the liver (*In vivo*) or by removal of genes, injection of the new material and reintroduction to the body (*ex Vivo*). Stem cells continue to be the longest lived cells as well as self renewing.

Gene therapy to relieve haemophilia FVIII and FIX has been successful in mice. While transfer in the larger mammal - dogs - has been successful (40 dogs have been cured of their haemophilia). There are marked problems such as development of inhibitors, and only short term expression of factor. Sheep models have been infused with FVIII and continue to produce human FVIII in their milk.

Lillicrap said clinical use of gene therapy is "10 years away".

Those of us around in 1979 might recall the then Minister of Health (Hon Frank Gill I think) saying NZ should not build a blood fractionation plant as gene therapy would be in general use "within 10 years".

## Prophylaxis

***By Lorraine Porter-Bishop***

Victor Blanchette (Canada) gave an in-depth overview of many different prophylaxis approaches used in Canada, Sweden, France and the Netherlands. Each of these countries uses a slightly different approach. In Sweden high-dose prophylaxis is started around the age of 1 year, when children begin to walk and before the first joint bleed occurs. The Dutch use an intermediate-dose approach. Prophylaxis is started after at least one joint bleed only 2x weekly for haemophilia A and 1x weekly for haemophilia B, intensifying according to the bleeding pattern. An indivisualised dose-escalation approach is used in Canada. All children begin will an early lose-dose of prophylaxis once a week. If bleeding exceeds certain criteria the frequency and the dose are increased. The French protocol is similar to the Canadian but tolerates some breakthrough bleeds.

He also talked about providing prophylaxis where safe factor concentrates are available. He looked at using primary prophylaxis to prevent any onset of joint damage rather than using secondary prophylaxis after joint damaged had already occurred. He went through a series of studies which had collected data on both primary and secondary prophylaxis and status of joints. Blanchette also covered the results of on demand treatment.

The question of when is the optimal time for starting prophylaxis remains unanswered and the optimum regimen is still under discussion. There is recognition that some patients with severe haemophilia experience very few bleeding episodes and so perhaps not everyone should be treated the same. Cost can also play a factor in different approaches currently being used.

## The Role of Prophylaxis in Bleeding Disorders

***By Richard Scott***

Erik Berntorp (Sweden) spoke on **Prophylaxis in VWD**. Around 37% of people with vWD have joint bleeds. There is only limited experience with prophylaxis in 37 patients in Sweden. They have one infusion per week, 45 weeks per year. Most of the patients on prophylaxis have Type 3 vWD. Those chosen for prophylaxis had joint bleeds and nose, mouth and GI bleeds were also common. Doses varied from 12 to 15u/kg of body weight. This treatment continued over varying periods from 2 -45 years. Children who were started on prophylaxis before the age of 5 years never developed arthropathy. Other smaller cohort studies has also confirmed the effectiveness of prophylaxis in vWD. There are two studies currently underway, a retrospective study and a prospective study involving 21 centres who have enrolled 83 patients so far. There is strong support of prophylaxis for those most severely affected by vWD, although there hesitation in some countries as there is no recombinant vWF yet available (only plasma-derived). There may not be a need for FVIII in vWF concentrates. He concluded that prophylaxis in vWD should be used more often.

Philippe de Moerloose (Switzerland) discussed **Prophylaxis in rare bleeding disorders**. There are no randomised trials supporting prophylaxis in rare bleeding disorders. All information available is based on small or even individual cases as prevalence may only be 1/1,000,000 compared to the 1/10,000 diagnosed with haemophilia. Rare bleeding disorders are difficult to diagnose and treat. In rare bleeding disorders intra cranial hemorrhage is more of a concern, and menorraghia and pregnancy is often an issue for women.

Primary prophylaxis is given to prevent life threatening bleeds and/or to prevent joint bleeds. It can be suitable for those with frequent bleeds in daily life.

Secondary or short term prophylaxis can be given during surgery and during pregnancy and child birth to prevent spontaneous abortion and post-partum haemorrhage.

If you have a rare bleeding disorder and you live in a developed country then prophylaxis may be appropriate. Product availability may be the issue, e.g. there is no factor V available. There is a risk of some side effects of treatment substitution. For example, finding the right balance between stopping bleeding and thrombosis (blood clot) can be tricky. Some rare bleeding disorders require an individual treatment program. Transexamic acid can be useful and cost-effective in many cases.

Factor X (10) deficient patients that are severely affected are good candidates for prophylaxis. Factor X has a long half life (20 -30 hours) so prophylaxis may only be needed two times a week.

Prophylaxis for those with factor XIII (13) deficiency is useful during pregnancy or during periods.

In general, bleeding history and severity (severe, moderate, mild) should be the indication of need. Prophylaxis should

be personalised to factor levels. Due to rarity it is important to belong to international registries.

Rolf Ljung (Sweden) finally gave a presentation on **Prophylaxis in Haemophilia**. In Sweden prophylaxis starts before a child with severe haemophilia turns 2 years old on the argument that arthropathy can have already started. In one study, Fisher et al., found that on-demand patients and those on prophylaxis used the same amount of factor, but that 12 radioactive synovectomies had been performed on those using on-demand therapy to ease joint damage whilst none had been need for those on prophylaxis. Those on on-demand treatment gradually needed more and more product and 10% had a life-threatening bleeding episode.

Everyone agrees that prophylaxis is effective and should be the treatment model for haemophilia. In Sweden, they start treatment early with a high dose. Directly comparing their high dose regimen versus a low dose regimen is distorted as many countries start treatment after the age of 2 years.

Canada initially use a 50 IU/kg once a week then moves to 30 IU/kg twice a week and 25 IU/kg three times a week. This is because of the venous access problem in young infants. They find that reducing venous access problems improves compliance. Around 40% stay on the once a week dose and the remaining 60% were escalated. By age 8, however, 50% have evidence of arthropathy on MRI.

The future of prophylaxis :

- Daily prophylaxis. Many teenagers in Sweden use daily prophylaxis.
- Tailored dosages. Using a low daily dose can reduce the amount of factor used by 40%. This is linked to product half life and avoiding the trough when factor levels are at a minimum before the next dose.

The regimen depends on the objective of treatment. Less intensive prophylaxis is sometimes used in developing countries because the goal is participation in society not complete elimination of arthropathy. Intracranial haemorrhage is 20 -50 times more frequent in a person with haemophilia than without ,so prophylaxis has an important part to play in the reduction of this risk.

His recommendations are that children are the first priority and to start once per week prophylaxis via venous access before the age of 2 years. If there is bleeding, increase the frequency as it is better to administer small doses frequently than one large dose.

## Haemophilia Care in the Future - What the Clinicians said

*By Mike Carnahan*

Dr David Lillicrap, Professor in the Dept of Pathology and Molecular Medicine, Queens University, Kingston, Canada was of the view that the issues dominating haemophilia currently are:

- Chronic arthropathy and intracranial bleeds affecting the very young and the very old.
- How to use prophylaxis to overcome musculo-skeletal disease, although its use is proven up to adolescence.
- Improvement to recombinant products by molecular modification to extend the half life, increases the specific activity, and reduces immunogenicity.

Dr Johannes Oldenburg, Professor at the Institute of Experimental Haematology, University Clinic, Bonn, Germany, talking on prophylaxis in Europe said:

- Prophylaxis in children continues to expand.
- Half-life and specific activity in children is not as good as in adults.
- Using prophylaxis in adults over 30 did not prevent arthropathy.
- Both WFH & WHO recommend the use of prophylaxis indefinitely.

Dr Glen Pierce, Chief Medical Officer, Biogen, Waltham, Massachusetts, USA is working on increasing the half life of factor IX from 18 hours to 47 hours.

Dr Margaret Ragni, Professor of Medicine, University of Pittsburgh, Pennsylvania, USA, said:

- Phase I & II trials are proceeding on development of rFIXFc, a long-acting, fully-recombinant Factor IX Fc fusion protein with a threefold increase in half life and a 24% improvement in recovery. See www.biogenidechemophilia.com.

Dr Jerry Powell, Professor of Internal Medicine, University of California, USA believes:

- More progress is being made in haemophilia than any other genetic disease primarily because of the preparedness of patients to participate in trials.
- Future care will result in patients spending less time at a factor level of 1% and below 1%.
- Future care will result in patients having less trouble with inhibitors.

Dr Katherine High, Professor of Paediatrics, University of Pennsylvania, USA, said:

- The future will be haemophilia care without the use of replacement blood proteins but with the use of gene therapy.
- Gene therapy continues to trial various vectors to transport the gene to the delivery site which may be large muscle or liver.
- Haemophilia dogs treated with gene therapy continue to express the gene after 10 years.
- In humans, the expression of the factor reduces over 14 weeks.
- Work is progressing on the use of small molecular drugs capable of reading through a "nonsense mutation".

Dr Jesper Haaning of California, USA, outlined some of the novel therapies presently being worked on:

- Reducing the dose volume
- Removing all human and animal protein from products
- Pegylating the FVIII molecule to improve the half life from hours to maybe seven days.





# NMO Training Workshop

*By Karl Archibald*

***Karl received a special Youth Scholarship from WFH to attend both the pre-Congress National Member Organisation (NMO) Training workshop, the Congress and the WFH General Assembly. Below are his reflections on the experience.***

**Tuesday – Pre-Workshop Day**

2.30 am and I am as awake as it gets. It is 5.30pm back home and my body clock is telling me so. 6 am and I am hungry the restaurant opens at 6.30. I lie there counting down the minutes… 6.07… 6.12… 6.20…9.30. Win, got some sleep. Got to get up, the restaurant stops serving food at 10am.

After massive breakfast I was keen to do nothing. It is officially a rest day before the workshop starts but pushing laziness aside we wandered down to a cafe near a petrol station with your friendly shotgun armed Argentine security guard outside and grabbed a unique style of Cappuccino. Ono lazzar cappuccino por favor? Creme senior? No gracias. My Spanish is slowly getting better. Argentineans' know their stuff. We have a couple of hours to kill so Augustus and I are informed that we should head to a place called Paila as it is good and well worth a visit... No it is not.

Walking through the area we are staying is an experience. It is supposedly not dangerous as it is middle-upper class neighbourhood, but apparently it's a bit like Moscow, just without the vodka. I walk around the place and do some more sightseeing.

Back at the hotel, we hit the couch, and watch some football . The game ends and we call it a night and I head to the room to write some more. Morning comes around and this is as far as I got.

Ciao,
Karl

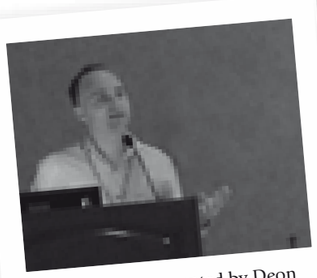
**Wednesday - First Day of NMO Training**

7.30am (10.30pm NZST) and I awake from my slumber. Deon is already half way out the door. "I'll see you at breakfast." I drag myself into the bathroom and shave off a week's worth of stubble, cutting my face in more places than I can count and head downstairs. Thank goodness for prophylactic treatment. Breakfast is well underway. I put the minuscule amount of bacon that is left onto my plate and grab a seat no sooner that I sit down a hand touches my shoulder and a deep Canadian accent bellows into my ear. "15min 'till we start Karol, better hurry up". Please mate. You have obviously not seen me eat. I inhale the rest of my breakfast, down a glass of juice and head to the seminar room with 12 min to spare...

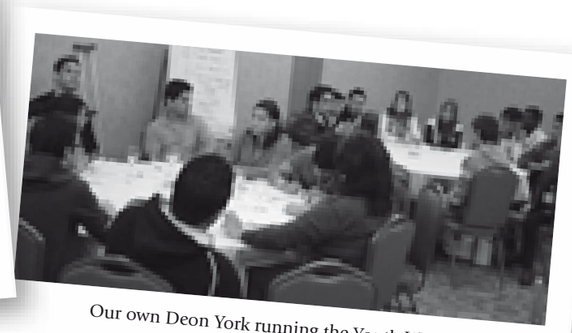
We are divided into groups, and I take my seat. Suddenly a strong and assertive voice enters the air, there is an instantaneous hush. "Welcome to the 5th global NMO training. My name is Mark W. Skinner and I am the president of the WHF." We are given a run down on the day's events and then set into a team building exercise - a quiz on bleeding disorders. My team came second. Deon's won. I decide the Kiwis are Awesome.

For the first session we are informed about what the WFH does, their mission statement "'Treatment for all', etc. History: Founded in1963, recognised by WHO in 1969 and has 114 National Member Organisations (NMOs), with 4 pending. The 4 additional countries were voted for and passed with a unanimous vote at the General Assembly (after the Congress). WFH now has more than half the countries in the world as members.

WFH is made up of an extremely large network of volunteers. They have 32 full-time staff and operate off a budget of just $4 million. We discuss their strategic plan for the next 3 years, some of which includes: treatment to emerging countries; continued and sustained treatment for established countries; enhance treatment for vWD and rare bleeding disorder; share knowledge and how to build a network of people through Integrated communication technology (ICT). These goals will help improve the lives of the 220,000 people with bleeding disorders identified so far, and the 440,000 suspected people with bleeding disorders (numbers as at 2008).



Youth issues presented by Deon



Our own Deon York running the Youth Workshop

We are passed over to Claudia Black, the CEO of the WHF, who gives us a brief overview of how her and her team work. She speaks about areas she is particularly passionate about such as product donation, establishing a WHF guideline for setting up a haemophilia treatment centres, which include increased product purchases, and establishing a national registry. They are also working towards enhancing access to people with haemophilia and rare platelet disorders. WFH have set a goal to have 88 treatment centres in 61 countries by the end of 2010.

Elizabeth Myles and Sarah Ford gave a presentation on Social Media, new strategies in communication and websites using latest interactive trends. This covered what the issues are today in terms of communication, how Facebook, Twitter, Linkedin and Flicker are the 4 major social media tools that are used by people and is what WFH are using to reach out to people from around the world in order to raise awareness and educate others.

They discussed how they find that marketing via social networking has a 92% hit rate and how people reach out with Micro Blogging, Blogs, Video sharing, live casting and music. It demonstrates how trends have changed in the last 10 years and how people turn to electronic means of communication over other forms and is much more effective than traditional forms

**Thursday – Day 2 of NMO training**

We gather around tables in mixed groups to discuss the finer points of conflicts and how to resolve them. This session, as the opening sentence suggests, is called Conflict Resolution. This subject is brought to you by Eric 'the pacifist' Stolte and Gordon 'I like a fight b'coz I'm Irish' Clarke. Eric was a co-opted member of the WFH Executive Committee, and Gordon was stepping down from the Executive Committee this year as he had served his full ten year term. Gordon and Eric are two appropriate people for this topic. We discussed case studies and strategic ways to deal with conflict should it arise. It was interesting to see the various forms and techniques that have been used by other NMOs. We were given 3 case studies to discuss and the groups come up with ways to answer each of these challengers. Overall I got a lot from this session.

We break for lunch and I do a washing run down to the drycleaners at the end of the block and get a whole supermarket bag done for 16 pesos (slightly cheaper than the in house cleaning ranging from $5-$60 pesos per item of clothing).

That afternoon we move into a youth plenary (by WFH standards 'Youth' is defined as people aged between 18 and 30). This interactive work shop is chaired by Deon York, who is a co-chair of the WFH youth working group, our HFNZ President (as you know) and now a lay member of the world Executive Committee. *Busy Man*! One Gordon Clarke co-chairs this workshop.

This was a brilliant session. It ran for over 2 hours and could have gone on much longer. Areas that we covered were motivators of youth, and how to maintain a degree of dedication by dividing time between voluntary work, study, work and social activities. We hear 5 youth talk about their local communities, ranging from developing countries, emerging, through to established. They spoke how each country worked with youth and developed a range of camps that met the needs of the group including social and medical aspects.

We break for tea and move into a Youth working group that Deon runs. We talk informally about what issues each local haemophilia community is facing when it comes to youth involvement, medical requirements, social and cultural issues and study. This was attended by 30ish youth from a variety of countries and the diversity of knowledge and experience was a great learning and development opportunity.

After the working group, a group of us met - Ali from Iran, Baiba from Latvia, Dawall from India, Uros from Slovenia, Masood from Pakistan and myself, Karl from New Zealand - and talked for a good couple of hours developing ideas and action points moving forward from the working group. A couple that stood out I will be looking at developing to assist HFNZ's youth.

Conflict resolution session

Twinning role plays





**Friday - Final day of NMO training**

6 hours sleep as a result of staying up late talking the night before, however, it was worth it and the good news! : Jetlag is over!

Candis Terpstra presented a session on Twinning with HOT and HTS for established countries. For those who don't know what Twinning is and what the above three letter acronyms (TLA) mean here is the answer: Twinning is when a Developed country partners (twins) with a Developing country for growth and support from the Haemophilia Organisation Training (HOT), and a Haemophilia Treatment Centre (HTS) when you have both the hospitals and the local foundation .

It is decided that *ROLE PLAYS!* are a great way of getting to know the negotiating process and twinning roles. We are given props to make it more interesting. Deon becomes a Canadian Viking and I am designated a tiara. Awesome. We get the process off to a great start and even though no one wishes to do it we get a lot of development out of it and learn how Canada has twined with Lebanon and what challenges and rewards they have received along the way with the relationship that they have built.

We break and head into our next Plenary which is a rap up and question and answer session for the World Federation Executive Committee. There is a variety of questions on sourcing their focus and ways that they help others. A range of countries thank the exec team, staff and every volunteer involved with World Fed on how they have helped make the lives of so many easer to live and help in so many different ways from education, medically, and networking.

The final dinner was a great evening rap up and everyone was into it. Some of the music that played was the type you'd play if you were in a club back home and wished to lose all your business (e.g. YMCA), however it was a great night.

I have included an assortment of photos from throughout the night and training for your entertainment. All stories are better with pictures...

Final Dinner at NMO Training

Deon representing HFNZ at the WFH General Assembly

WFH Executive Committee at General Assembly

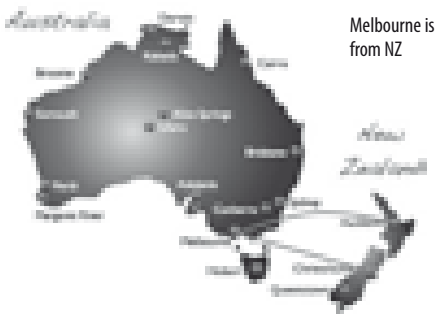
# WFH News

## Deon York first NZ lay member of WFH Executive

At the WFH General Assembly held on 15 July, HFNZ President Deon York was elected into a position on the WFH Executive Committee. He is the first New Zealander to serve as a lay member on the Executive. This is a great honour and a fantastic achievement, for Deon and HFNZ. It clearly shows the high esteem Deon is held in by members of the global bleeding disorder community.

Deon already serves on a number of WFH committees and now joins the thirteen other medical and lay members on the Executive committee.

Congratulations Deon!




Melbourne is only 3 hours from NZ

## Melbourne for 2014 World Congress

Haemophilia Foundation Australia is delighted to announce that the members of the WFH General Assembly voted that Melbourne will be the host city for the 2014 World Hemophilia Congress.

HFNZ were excited to support the bid for Melbourne as having a World Congress so close to NZ should make it easy for many New Zealanders to attend and experience this unique educational opportunity. The Congress will bring together delegates from around the world to take part in the program, which will cover medical, multidisciplinary and laboratory science issues, with topics of special interest for people with bleeding disorders, members of national haemophilia organisations and health professionals.

**Hemophilia 2012 will be held in Paris, France.**

# Dates to Note

**3-5 September**
Central Region Winter Escape
Masterton

**18 September**
Northern Region Movie Night

**25 September**
Children's Education Workshop (Central)
Wellington

**28 September**
Children's Education Workshop (Midland)
Hamilton

**30 September**
Children's Education Workshop (Northern)
Auckland

**2 October**
Children's Education Workshop (Southern)
Christchurch

**24 October**
Armageddon Masquerade Ball
Logan Campbell Centre, Auckland
All ages - Proceeds go to HFNZ

**5 December**
Northern Region Christmas Party

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

ARMAGEDDON

## "Have plans for Labour Day Weekend? In the Auckland area?

Want to experience the largest fantasy and gaming event in the southern hemisphere?

HFNZ is looking for volunteers to work at Armageddon Expo at the Auckland ASB Showgrounds.

Contact info@haemophilia.org.nz for more info.

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

Jeff Oliver Print - Whangarei