National Haemophilia
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WFH 2012 WORLD CONGRESS

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HAEMOPHILIA AWARENESS WEEK

Haemophilia Awareness Week is an opportunity for Haemophilia Foundations and other organisations, as well as individuals and families to take part in a campaign and activities to raise awareness about haemophilia, von Willebrand disorder and related inherited bleeding disorders throughout Australia. This year, the week of 7-13 October has been set aside and we encourage all our supporters to participate. The theme this year is *"Achieving your dream"*.

There are many ways you can help us promote Haemophilia Awareness Week:

- Take part in Red Cake Day
- Set up a stand in your workplace, school, hospital or library
- Hand out promotional items in your local area
- Assist your local Haemophilia Foundation during the week
- Organise a casual clothes day at your workplace or school
- Organise a luncheon, sausage sizzle or morning/afternoon tea.

Promotional items such as stickers, tattoos, slap bands, posters, and colouring-in sheets are available for schools, work places, hospitals and community centres. To place an order for items (free of charge), download an order form from our website www.haemophilia.org.au (click on the Awareness Week logo on our homepage to be directed or look under Events and Awards) or email hfaust@haemophilia.org.au. Please note that stocks are limited.

For further information about Haemophilia Awareness Week or to order promotional items, please contact the HFA office 03 9885 7800 or email hfaust@haemophilia.org.au

RED CAKES CAN CHANGE LIVES!

It's true. They can. That's why HFA is calling on our friends and supporters to help us celebrate Haemophilia Awareness Week by taking part in our first Red Cake Day!

It's easy…all you have to do is bake some delicious red cakes or cupcakes, decorate them, and share them with your friends or work colleagues in exchange for a donation or a gold coin.

You could take them to work, school, have a cake stall or simply host an afternoon tea with your nearest and dearest family and friends. Not only will you be having a delicious morning tea but you'll be helping raise funds and spread the word about haemophilia and other inherited bleeding disorders!

Then, simply send the donations to Haemophilia Foundation Australia. All funds raised will go to a range of programs and services ran around the nation.

Want your cakes to look extra special? Why not order some of our specially made H logo cupcake transfers? They're fully edible and will turn your red cupcakes into something truly fabulous! Check out the order form for more information. But hurry, we only have a limited number available!

Download an order form from our website www.haemophilia.org.au (click on the Awareness Week logo on our homepage to be directed or look under Events and Awards) or email hfaust@haemophilia.org.au.

WFH 2012 WORLD CONGRESS

The XXX World Federation of Hemophilia (WFH) 2012 World Congress was held in Paris, France, from Sunday 8 July to Thursday 12 July 2012.

In this issue of *National Haemophilia*, HFA delegates at the WFH Congress describe their experiences and give reports of meetings, workshops and presentations during the Congress.

Natashia Coco is Haemophilia Foundation Australia Development Manager

THE EXHIBITION EXPERIENCE

Natashia Coco

MELBOURNE 2014 – WE ARE NEXT!!

In Paris it was time to promote Australia and encourage delegates to come to our country for WFH World Congress 2014. Our booth was one of the busiest stands during the Congress, and we created a real Australian feel complete with a koala, kangaroo, Australian native floral arrangements and some of our well known foods for people to try. Many people were uncertain about Vegemite but tried it anyway with a cracker, and they also enjoyed sampling Furry Friends chocolates, Melbourne 2014 pins, Australian bird bookmarks and information leaflets about Australia and Melbourne. Most were interested in how to get to Australia, the likely costs, distances and touring options. As a further incentive we had a competition for a free flight to Melbourne in 2014.

We received some great feedback from people who are looking forward to coming to Australia, and we hope to see them all.

So save the date – 11 to 15 May 2014 in Melbourne!

Natashia Coco and Jurarat Suriyathai from Thailand at the HFA Booth

Gavin Finkelstein is President, Haemophilia Foundation Australia (HFA)

FROM THE PRESIDENT

Gavin Finkelstein

We always seem to have a busy schedule at HFA, but the last three months has been exceptionally hectic for several reasons.

WORLD CONGRESS

In the last few months we have been preparing for the recent World Hemophilia Congress in Paris. Not only was the meeting important to us because it is such a valuable medical and scientific meeting that brings all the experts together in the one place, but it is a great networking meeting for people with bleeding disorders and their families and we try to support and encourage as many Australians as possible to attend. The attractive location of Paris was probably encouragement enough, and many Australians went under their own steam.

As it was also the last meeting before the World Congress in Melbourne in 2014 HFA had special responsibilities. We wanted to be sure the people involved in the planning and organising work towards 2014 had a good understanding of how congresses operate. They also needed to attend several congress related meetings.

HFA had a booth in the Congress Exhibition to promote the 2014 Congress in Melbourne. Our booth had been designed to promote Melbourne as a great meeting and tourist destination and focussed on the opportunities for travel to other parts of Australia and the region. There was great interest and it was exciting to observe visitors come to the booth to ask questions about visiting Australia. It was even more exciting to see HFA delegates, members of State/Territory Haemophilia Foundations and health professionals from around Australia proudly wearing polo shirts to promote the 2014 Congress.

We will need a huge team of volunteers and supporters to ensure the 2014 Congress is a success for both the World Federation of Hemophilia (WFH) and HFA, and we plan to work with all stakeholders to make sure it is indeed a success. The HFA Executive Board began to develop more tangible plans for this at its recent August meeting and there will be much more on this as time moves on.

Jurarat Suriyathai presenting at World Congress

In this newsletter we have included the experiences of some of the delegates at the Paris Congress and some of the important information they gathered about the developments and future horizon for the care and treatment of people with bleeding disorders around the world, and the ways we might measure treatment outcomes and success. We have many challenges and I think we will see changes ahead – in terms of new treatment options for many, a cure for some, and different approaches to the financial challenges for governments who are paying for treatment and care. I believe it will be very important that we are engaged in these challenges and play our part – we know the benefit of clotting factor and how access to appropriate supplies for our bleeds has improved our lives. We need to be able to demonstrate this difference with evidence that is understood by policy makers in hospitals, and governments at all levels.

It was great to see the broad participation of so many Australians in the Paris congress program, including people with a bleeding disorder or their family members, health professionals and other volunteers who had been invited to give presentations, chair sessions or present posters. I was pleased to have worked with the Haemophilia Centre of Western Australia team to present a poster "How the concept of peer support has been used in Western Australia to improve healthcare outcomes for people with bleeding disorders". The poster display area at the congress was vibrant and it was great that so many Australians made the effort to prepare posters about new developments in their area of expertise.

We were also pleased that Jonathan Spencer, HFA Vice President represented HFA at the Global National Member Organisation Training before the Congress. You will hear his insights in this newsletter.

CLOSING THE GAP

WFH has been able to make huge improvements around the world in countries where treatment had been previously unavailable or inadequate. This is primarily through WFH partnerships and programs such as Global Alliance for Progress (GAP), and other WFH development programs such as haemophilia centre and haemophilia organisation twinning programs around the world. Although HFA's official twining partnership with the Thai Patient's Club in Thailand is ending, we remain close to our Thai friends and it was good to be able to meet with Thai delegates when we were all in Paris to discuss further work we can do together. We were delighted that Ms Jurarat Suriyathai was invited to give a presentation at the Congress on the outreach programs in regional Thailand which have identified new patients and helped improve access to treatment and care to people in very isolated regional and rural areas of Thailand.

During the Congress the message was loud and clear, that for all the progress around the world, there is still much work to be done. WFH has launched a major fundraising campaign, the Cornerstone Initiative, to raise funds for development programs that might extend care and treatment to countries currently at the lowest end of the economic spectrum. As a founding member of WFH, HFA was pleased to be able respond with a donation towards this new program.

WFH GENERAL ASSEMBLY

After the Congress I represented HFA at the WFH General Assembly. This meeting is held every two years after each Congress and the business includes presentation of financial and executive reports as well as the election of members and office bearers to the WFH Executive Board, and the selection of host countries for future congresses. After many years of service Mark Skinner finished his term as President, as did Associate Professor Alison Street (Vice President Medical) and Rob Christie (Vice President Finance) and several others who have generously volunteered their time and expertise to the work of WFH. WFH is an amazing organisation, and its achievements largely stem from the generosity of its volunteers, supported by a committed staff. The volunteers come from all parts of the community and from many different countries.

I congratulate Alain Weill (from France), who is the incoming WFH President, and Dr Alok Srivastava (from India), Vice President Medical, and Eric Stolte (from Canada), Vice-President Finance. We look forward to our ongoing work together.

HFA FUNDING NEWS

We recently negotiated a new funding agreement with the Department of Health and Ageing (DOHA) for the next three years. This agreement is one of the two we have with the Department and it covers a significant part of our administration and governance activities such as our annual Council meeting and Board meetings throughout the year. The grant is tied to our work to represent people with bleeding disorders and gives us the capacity to undertake our work.

Most of HFA's work is funded through its fundraising program, but we would be unable to operate without our government grants that help fund our operational expenses, education activities and provide support to health professionals groups. They also include specific project grants such as for the Youth Project, which are available from time to time.
We were grateful that over the last year we also received a DOHA grant for the Youth Project which had also been partly funded with a donation from a charitable trust. This grant was extended in May for the youth leadership and mentoring components of the project, and will enable us to fund our Youth Project for a further year.

We are nevertheless increasingly challenged by the fundraising climate and readers will have heard that many organisations like HFA have struggled to meet their targets. We too have been challenged, and we have to make choices all the time about how we spend the Foundation's money. Unfortunately we are not always able to do the things we want to do, or need to do, in a timely way. This can be frustrating for us. The HFA Board and staff have been working hard to review and evaluate our fundraising program and to put plans in place to make sure we have the funds available for our short term needs and for the future. We see this as a normal part of our strategic planning, to ensure our organisation remains robust and responsive to the needs of our community.

Jurarat Suriyathai and her co-presenters at World Congress

Gavin Finkelstein and Sharon Hawkins at their World Congress poster

Jonathan Spencer is Haemophilia Foundation Australia Vice-President
Suzanne O'Callaghan is Haemophilia Foundation Australia Policy Research and Education Manager

NMO TRAINING

Jonathan Spencer attended the 2012 WFH Global National Member Organisation (NMO) Training on behalf of HFA. He spoke to Suzanne O'Callaghan about the Training and his experiences there.

S: Where was NMO Training this year?

J: The Training took place over three days at the Hotel New York Convention Centre at Disneyland Paris, just before World Congress. Unfortunately we were too busy participating in the workshops to go on any rides!

S: What was your overall impression of NMO Training?

J: There were over 100 delegates from all around the world - a great mix of younger and older delegates and from established and emerging NMOs. The enthusiasm of the delegates from emerging NMOs was really energizing, as well as the absolute passion of individuals to improve the situation in their own countries and worldwide. Seeing things firsthand is very emotionally confronting. It gave me time to pause and reflect that that it's just through the luck of being born in Australia that we have lived with such a good health system and the access to treatment product and care which is only beginning now in developing countries. You not only hear the stories about what's happening in emerging countries, you see the evidence in front of you. Personally, I felt very motivated to move forward to close the gap of treatment and care between developed and developing countries. It can mean the difference between life and an early death in some countries.

I met some marvellous people there - amazing advocates, and they know how to party! They get fully involved in everything that they do.

S: What were the key sessions?

J: The object of NMO Training is not just to bring people together to share stories, but to build the skills of NMOs, and to develop the skill expertise and awareness of people to advocate in their country. In the workshops WFH separated the groups into emerging and established NMOs and into English-speaking and Spanish-speaking groups. Emerging and established NMOs have different issues to deal with and this helps the groups to focus on developing skills that are specific to the issues they encounter. I was in the group for established NMOs which concentrated on building advocacy skills. There was a spread of younger and older people and of experience and skills. Gordon Clarke and Amanda Bok facilitated the group very ably and were able to bridge the differences, cover a lot of ground and bring the group forward together at a very high level.

S: What sessions stood out for you?

J: I was presenting at the Advocacy Strategies session, so my memory of the event is a bit coloured by that. I spent quite some time in the weeks beforehand preparing with Gordon and Amanda and the other presenters.

With Radek Kaczmaerek, a really delightful switched-on guy from the Polish Hemophilia Society, I co-presented "Building and Managing Reputations" in advocacy strategy. My key message was to use but also protect your reputation. Be aware that the environment can change and you need to adapt; sometimes quickly to take advantage of opportunities. In other times, perseverance may be the best strategy. It might seem complicated, as one strategy may not fit every circumstance. But, a perhaps a universal message is to always act cohesively – for example, working with all levels of government and keeping your community well-informed.

Apart from that, one of the most memorable sessions for me was the screening of the film *Bad Blood*. It's largely an historical film and has to be understood in the context of its time, but it really highlighted that you have to be ever vigilant about the risks and issues that might arise; we are very much at the vanguard and must not accept the status quo without questioning it. It was very important to have the discussion afterwards to put it in perspective as I think most of us at first have a very emotional response to it, and then need to think through what it means today and how we move on from it.

There also seemed to be a strong focus on youth. A lot of young people attended, and it was great to see what the issues are for young people now. It seemed to me that our challenge is to get our talented and switched on young people involved here. People were very interested in the HFA youth project and to see how it moves from establishment to being able to drive itself.

S: What do you think was the value of NMO Training for HFA?

J: The NMO Training is a great opportunity for HFA to get involved face-to-face – to meet others with the same issues and dilemmas and connect. At a national level, it is important for HFA as an established NMO to be 100% involved with the bleeding disorders community around the world and in our region. We have more resources – we have the skills and we have the people, and we can't just sit back and think about what is happening here, and in our local area; we need to be aware of what's happening globally. We need to become fully involved with our world community as best we can. We do that already through WFH programs and through Twinning, but we all need to see what opportunities we have to be involved.

People will have an opportunity to do this face-to-face in the 2014 World Congress in Australia. At a personal level, you think you have a rare bleeding disorder, and it is an amazing thing to meet someone who walks just like you, who has the same issues and the same dilemmas. In your local area you can feel very isolated. At these events, you realise there are many other people with bleeding disorders who have similar experiences – you can see that you are not alone.

Ann Roberts is Treasurer, Haemophilia Foundation Australia

TREATMENT ADVANCES AND CLOSING THE GAP

Ann Roberts

I was greatly honoured to be able to attend the WFH 2012 World Congress. It has renewed my determination to work towards improving the care of people with haemophilia and other bleeding disorders in Australia as well as all over the world. Hopefully I will be able to be involved with the organisation of the upcoming Australian World Congress to be held in Melbourne on 11-15 May 2014.

I attended a number of sessions but was particularly impressed with World Federation of Hemophilia (WFH) continuation of the "Closing the Gap" program. This campaign relates to bringing basic care to the poorest countries throughout the world.

WFH PRESIDENT'S ADDRESS: CLOSING THE GLOBAL GAP – ACHIEVING OPTIMAL CARE

Mark W Skinner, USA

Haemophilia Treatment Advances

Speaking at the opening session of the World Congress, WFH President Mark Skinner spoke about the development of treatment since 1963 when the WFH was founded.

The last 50 years has brought tremendous advancement in haemophilia care. The WFH role in this began when a group of 12 countries, one of which was Australia, formed the World Federation Hemophilia which has now grown to include over 120 countries. This has resulted in improved care throughout the world.

Mark Skinner reminded us that in the 1960s, fresh frozen plasma (FFP) was the principal therapy available for treatment of bleeds but is still today used by some countries. In 1964 Dr Judith Pool was responsible for a method of preparing concentrated factor VIII from the thawing FFP, which changed the course of haemophilia care.

In the 1990s the development of recombinant analogues FVIII, FVIIa and FIX again changed the care and quality of life for people with bleeding disorders in the developed world. Of course, these advances also brought to the community a huge cost in the form of HIV and hepatitis C, the results of which are still being felt today.

Another advance in haemophilia care came with the multidisciplinary comprehensive care model as a public health care model which again brought tremendous benefit to patients who were able to access Haemophilia Treatment Centres.

Today another revolution is just around the corner with new treatment from viral inactivated cryoprecipitates , generic versions of current therapies, long life products, and with the concept of gene transfer, a "cure" may yet become a reality.

These advances have not been and are not available to everyone with a bleeding disorder particularly in emerging countries.

Data collected by WFH in 2010 show that there are now over 257,000 people who have been diagnosed with a bleeding disorder and entered in national registries around the world.

The WFH GAP program goal in 2003 was to identify 50,000 new patients. In the eight years to 2010 WFH have identified nearly 58,000 new people with bleeding disorders.

But still 75% of people with a bleeding disorder do not receive adequate care or any care at all.

Mark Skinner asked us all the question - what will it take to close the gap?

The WFH GAP Program continuation

WFH is committed to reaching Treatment for All and Mark Skinner outlined the key initiatives for continuation of the GAP program for the next decade 2012-2022 to achieve this:

- Increase the number of people diagnosed with bleeding disorders by another 50,000 people
- 50% of which would come from the most impoverished countries.
- It will take time, volunteers, and, most importantly, financial resources.

A key element is the Cornerstone Initiative 2013-2022.

This program will target countries and regions where even diagnosis of a bleeding disorder is futile given that there is a complete lack of access to even basic care. This will be achieved by:

- Developing diagnosis capacity with those targeted countries
- Providing basic training in bleeding disorder management
- Building patient organisations
- Aiming to close the gap by 2022.

Dan Credazzi is Haemophilia Foundation NSW President and HFA Executive Board Member

FROM PARENTS AND FAMILIES TO INTERNATIONAL DEVELOPMENT

Dan Credazzi

FAMILY ISSUES: FROM CRADLE TO TWEEN

Chair: Richan Mochan, India

Richan Mochan, India; Dragan Micic, Serbia; Traci Marshall-Dowling, Ireland; Pam Walton, Canada

Richan Mochan, the first speaker from India, discussed the general lack of quality treatment in India but that progress was being made as more factor became available. She highlighted the stigmatization and victimization of carriers in the Indian culture which has a devastating effect on the families affected by bleeding disorders. Not only is the condition a problem for the both the person with haemophilia and the family, but also the mother may be 'blamed' for the condition and families often fall apart as a result. This leaves people affected by haemophilia much worse off as they lose a lot of family support.

The second speaker from Serbia, Dr Dragan Micic, discussed 'maximising the health of boys with haemophilia'. Dr Micic emphasized the need for a different approach at different ages and the primary protective implement being the helmet. Serbia has a population of 11 million people and three treatment centres across the country. In a wonderful development, in April of this year, Serbia legalized home treatment self-infusion. Prophylaxis is now available for people from 2 to 18 years of age and as a result the situation is improving for people with haemophilia in Serbia.

Pam Wilton, from Canada, spoke about 'how to deal with diagnosis'. She is a nurse and herself carries the haemophilia and von Willebrand disorder (VWD) genes and is the mother of a son with haemophilia A and VWD. She commented that in her experience, both at home and at clinic, "it could be worse" is said a lot. From Pam Wilton's perspective, the "Kubler-Ross Grief Cycle" is very relevant to bleeding disorders and in particular news of a haemophilia or VWD diagnosis. Regardless of the actual severity of such 'news', the impact is manifested in the individual's perception of the "negative" event. The stages of the grief cycle are: shock, denial, anger, bargaining, depression, testing and finally, acceptance. As a nurse she thought that it was common when assessing people's conditions to start from the worst assumption and work backwards from there. Working alongside the Grief Cycle, this conservative approach aims to rule out the worst things with solid diagnosis and treatment on the journey to acceptance.

CLOSING THE GAP: CONTINUUM OF DEVELOPMENT

Chair: Bruce Evatt, USA

Bruce Evatt, USA; Arafat Awajan, Jordan; Ampaiwan Chuansumrit, Thailand; Monthon Suwannuraks, Thailand; Maria Satti, Sudan

I was particularly interested in this session on the GAP programs and development as the HFA Thai Twinning partners, Drs Ampaiwan Chuansumrit and Monthon Suwannuraks made a presentation about their development work in Thailand.

Bruce Evatt began the session with a discussion about the early twinning programs which gave rise to the GAP program and mentioned that Dr. Kevin Rickard, the HFNSW Patron and former NSW Haemophilia Centre Director, playing a pivotal role in the establishment of these early programs. The emphasis in these programs has been to create the right type of program for the given circumstances in a particular country. Each situation will be different due to the sheer amount of variables (i.e., culture, knowledge, factor availability, legal constraints, government support).

The Thai speakers commented that one of the best things they have created in Thailand is their new 24-hour Bleeding Disorders hotline. This has proven to be a great resource for families and people with haemophilia and has raised the general understanding of treatment options and lifestyle issues.

Dr Satti from Sudan presented on the situation in Sudan, which is very grim for people with haemophilia. Death rates are very high, particularly under the age of 4 years, where many are at risk of death due to head trauma or similar serious incidents. Sudan has only one treatment centre, located in Darfur, but it is so far for most people to travel that it is impossible for them. In effect, there is no access to treatment; the treatment that is available is plasma-derived and the safety of this is not guaranteed. With the help of their Twinning partner, Canada, Sudan has now created their first diagnostic laboratory. This has made a great difference as it enables them to diagnose bleeding disorders more accurately.

PARENTS EMPOWERING PARENTS: A GLOBAL PERSPECTIVE

Ed Kuebler and Danna Merritt, USA

This train the trainer Parents Empowering Parents (PEP) program was developed in the USA and involves nurses and social workers teaching parents to be facilitators as well as participate in the learning. They have developed a well-tested ten step process for this program, with both Facilitator and Parent Manuals. Items covered in this program include:

1. Basics of bleeding disorders
2. Child development
3. Compassionate discipline
4. Fine tuning behaviour management
5. Understanding your unique world view
6. How thoughts and feelings affect parenting
7. Building self-esteem
8. Understanding the process of communication
9. Refining communications skills and conflict resolution
10. Parenting Styles

I particularly took note of one parenting "rule of thumb" given as an example, which was the "4 to 1 rule": that is, four positive things said for every one negative thing said per day. I felt this is a good rule of thumb to live by. Other examples from the PEP training were the "Golden Family Rules": Don't hurt yourself, don't hurt others and don't hurt your environment.

In 2007 the PEP program came to Australia and a number of Australian haemophilia health professionals and parents received the training. I would be very interested in following this up with further training sessions in NSW and other states.

Jonathan Spencer is Haemophilia Foundation Australia Vice-President

WOMEN, YOUNG PEOPLE AND HAEMOPHILIA CARE ECONOMICS

Jonathan Spencer

WOMEN'S VOICES

Chair: Maureen Spilsbury, Australia

Murielle Pradines, France; Baiba M Ziemele, Latvia; Shahla Sohail, Pakistan; Silvina Grana, Argentina

Sometimes you make a fortunate mistake; or, maybe fate lends a hand! At the recent 2012 WFH Congress in Paris, I thought I was attending a session on "Haemophilia and Ageing" – probably important for a person with a bleeding disorder at my age; but, unknowingly, the venue had changed and instead I found myself in the sitting in the midst of some amazing stories, simply told by truly inspiring women.

I wondered if I had ever really understood the courage of women carrying the haemophilia gene before I heard Murielle Pradines share her family story. I was certainly not aware of the incredible social impact of a bleeding disorder on Shala Sohail in Lahore, who later became a successful physician and mother in London; nor was I aware of the 'sticky floor', as explained by Silvina Grana, psychologist. Related to the more familiar 'glass ceiling', the 'sticky floor' suggests it is accepted that a mother and carrier of the haemophilia gene will provide informal care for a child with haemophilia, under a strong sense of moral responsibility and to the possible detriment of her own well-being. Using humour and everyday experiences, Baiba Ziemele's laconic style portrayed a vivid story of family strength built on the characteristics (good and bad!) inherited from her grandmother. The collective voice of such intensely personal stories displayed a strength and acceptance that was previously unknown to me.

For me, this new perspective on bleeding disorders was the most memorable of the Congress. I didn't get to the session on ageing, but I was not at all disappointed. I gained so much from these stories.

THE ECONOMICS OF HAEMOPHILIA CARE

Chair: Declan Noone, Ireland

Angelika Batorova, Slovak Republic; Ricardo Carlos Gaitan-Fitch, Mexico; Daniel Anberg, Sweden; Keith Tolley, UK

The new priorities of safety, availability and affordability are placing haemophilia health care under intense scrutiny. I attended an extremely relevant session that demystified the economic assessment of health and haemophilia care. Costs are rising, new and improved drugs are coming to market and previously unconsidered general population issues such as ageing, obesity and diabetes are being studied with haemophilia. While we welcome an emerging more holistic approach to haemophilia care, a key role exists for established patient organisations to support our members and continue to advocate for a comprehensive care beyond the treatment successes previously won. Discussions about treatment are increasingly based on the value of the treatment, but, to date, there is limited objective data on short and long term consequences on which to base conclusions. More that ever, economic justifications are a necessary advocacy dimension, particularly as health budgets become more constrained.

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Keith Tolley explained that a Health Technology Assessment (HTA) may be undertaken to assess the cost-effectiveness, cost utility and cost benefit of new technology and drugs. The 'value added' by such developments is assessed by considering the incremental cost per Quality Adjusted Life Years (QALY) for a patient or patients. Relevantly, a recent study indicates that prophylaxis is more cost effective than on-demand treatment when QALY indicators are assessed.

HTAs may be used to assess developments to address current issues in haemophilia treatment today such as new longer half-life drugs, and dealing with inhibitors, and the cost benefit vs the cost of prophylaxis.

It is clear that well-organised national data registries are increasingly important for accurate data to undertake research and promote better product usage. A 2005 WFH Guide and more recent European research support the creation of national patient registries to provide equal access to modern treatment and nationwide care.

YOUTH – PREPARING FUTURE GENERATIONS

Chair: Dorothée Pradines, France

Deon York, New Zealand; David Pouliot, Canada; Diego Gavidia Huanay, Peru; Dorothée Pradines, France

We have all probably heard about the benefits of involving young people in our organisation.

I came to this session asking myself that, with so many benefits, why is it hard for patient organisations to engage with young people? The presenters had some very interesting perspectives on these issues.

While it's possibly counter-intuitive, strong organisations sometimes have an apathetic youth group as young people often do not perceive a need for their involvement. Further, experiences across the world suggest that, while young people experiencing difficulties are likely to become involved in programs, young people who do not feel affected by their bleeding disorder are less keen to attend activities and learn about their National Member Organisation. Certainly, there are many challenges and pressures on youth for their time and energy and other causes they feel strongly about and become involved in to advocate to achieve change.

So, what can be done to get more young people involved in their patient organisation? While using new media methods, such as facebook, blogs and YouTube, may help with communication and strengthen communities, a few simple suggestions may bring all closer together:

- Ask, don't wait for volunteers
- Have fun first, educate second and support transition to leadership
- Move young people to the centre of NMO activities with special positions, if necessary, and delegate responsibility to them.

Targeted programs for young people have also been successful - WFH operates a Youth Fellowship Program and maintains the Susan Skinner Memorial Fund Scholarship for youth. In New Zealand, of 12 scholarships offered to young people, nine have remained involved with that Foundation. Locally, HFA has begun work on its funded Youth Project to better engage with young people and has created a working group of young people who are involved with the development of a youth program. An important milestone for the program is the recent release of its youth-designed website, Factored In, at www.factoredin.org.au.

Dr Liz Bishop is a community member and a Lecturer and Research Fellow at the Michael Kirby Centre for Public Health and Human Rights at Monash University, Melbourne, and in 2012 presented at the WFH World Congress in Paris and the World Congress of Bioethics in Rotterdam

A SPEAKER'S PERSPECTIVE

Liz Bishop

It was hugely exciting to receive the news early this year that Haemophilia Foundation Victoria (HFV) were helping me to go to Paris to the World Federation of Hemophilia Congress. I booked my ticket to Paris the next morning. Within days I had also submitted an abstract to share my experience as a mother of two boys with severe haemophilia A and my knowledge as a medical ethicist.

My excitement was compounded when my abstract was accepted as a poster presentation and HFA were also able to help me pay my way to Paris. To my even greater delight the World Bioethics Congress also wanted me to speak at their conference two weeks earlier in Rotterdam. But how would I manage three weeks away from my family having never left them for more than the odd night before? I began to calculate the logistics.

The Bishop children with Hamish and Andy in London

Photo: Hamish and Andy's Euro Gap Year

Sadly, it did not occur to me to check the timings of either conference before enthusiastically accepting. It was my mother who pointed out that of the three weeks I would be away, two would be school holidays. My mother has not been well, my husband travels often for work and we have no other family support. In the end we decided the only option was for our three kids to come with me.

Armed with letters from the wonderful Royal Children's Hospital treatment team, three weeks supply of product, a poster in the most cumbersome and heavy tube Officeworks had available and two suitcases we set forth.

WORLD BIOETHICS CONGRESS ROTTERDAM

Rotterdam is a skateboarder's delight (according to 9 year old Hamish rather than to the Dutch cyclists whose dedicated lanes he was negotiating). I spoke with pleasure, and hopefully some enlightenment, to the audience of the benefits of family-centered care for children with chronic health conditions (and haemophilia in particular) requiring ongoing association with hospitals.

Our factor supply came in handy after an unfortunate bike lane incident in which, I suspect from the hand gestures but could not confirm from my minimal grasp of German let alone Dutch, that skateboards are not welcome in bike lanes. So too did our hard suitcase in lieu of crutches as we negotiated trams, buses and planes to make it to London.

LONDON

In London came the highlight of the trip (for the kids who obviously did not attend the conference in Paris). We went to watch the patron of HAUX (the Haemophilia Auxiliary of the Royal Children's Hospital) Hamish Blake in action with his best friend Andy at the Lord Stanley Hotel.

For those of you familiar with the steps, platform changes and Mind the Gap calls of both the London Underground and the Paris Metro, you will understand how handy it again became to have a firm wheely suitcase to cart our 15 year old, this time, around the labyrinth. My tips for travelling with boys with haemophilia around this system would not only be the hard wheely suitcases, limited luggage, backpacks and a sense of humour but, equally, a preparedness to accept the help of strangers. This came in surprising abundance – from both Londoners and the French – and we were shown great generosity.

WORLD CONGRESS PARIS

Through the streets of Paris and the depths of its Metro I wore with great pride the bright blue polo shirt supplied by HFA in honour of the 2014 World Congress in Australia. My expectations of the Congress itself were mixed. I was unsure what value it could have to me, let alone me to it.

Initially I introduced myself as just a parent. By the end I had shared the stories, experience and knowledge of many. My fondest moment was when the poster I had prepared brought tears to the eyes of another parent as we shared, momentarily, our stories. I was overwhelmed by how fortunate we are to have the access to treatment and trained medical professionals we have in Australia, which together with HFA and our various state associations such as HFV, represent our interests and positively agitate for change.

I have come away with a personal commitment to help boys with haemophilia and their families in other less fortunate countries to achieve equal benefits from progress within the haemophilia community. And, incredibly grateful for the experience. It took me 15 years to attend a conference and I exhort you all to make the most of an opportunity to experience one. Hopefully Melbourne 2014!

Jenny Lees is HFACT Secretary and convenor of the HFACT Women's Wisdom Group. She received some funding from HFA to attend the World Congress

CONGRESS – THE PERSPECTIVE OF A FIRST-TIMER

Jenny Lees

The WFH 2012 World Congress in Paris is the first world congress that I have attended. It was an incredible experience that taught me much and left me with a renewed appreciation of the standard and value of the conferences we have in Australia every two years.

There were over 5,400 people from 130 countries – people with bleeding disorders, carers, parents, spouses, doctors, nurses, physiotherapists, dentists, surgeons, counsellors, social workers, researchers, etc., all mixing and willingly sharing experiences, problems, successes, treatment results and research findings. I had the opportunity to chat to the Murielle, the convenor of the French women's group, about the role her group plays in France and about our Canberra Women's Wisdom group. The French group started as an opportunity for mothers and wives to chat and has grown in size, influence and acceptance. They have developed a training program that they deliver throughout France.

It was not all conference sessions and poster viewing; there were also opportunities to relax. The cultural show on Tuesday night featured a number of acts from a local circus school. The ostrich act by the contortionist was spectacular. The conference dinner at the Pavilion Dauphine was also an enjoyable night, complete with Australian wine!

The take-home messages for me were:

- Australia is a lucky country and we have to work to make sure it stays that way
- Long acting factor is close
- Gene therapy for factor IX is close
- Forget elbow replacement as the replacements do not weight bear!
- It may be useful to include the concept of chronic disease management in discussions about comprehensive care for haemophilia centres
- There is still a long way to go to close the treatment gap for bleeding disorders around the world.

Andrew Atkins is Clinical Practice Consultant at the Haemophilia Treatment Centre, Royal Adelaide Hospital

A NURSING PERSPECTIVE

Andrew Atkins

Andrew Atkins and Australian haemophilia nurse colleagues exploring Paris

It was always going to be good! The World Federation of Hemophilia (WFH) Congress in Paris in July was a thoroughly enjoyable event, and the 5,400 participants all seemed to enjoy the presentations and the city from the Opening Ceremony on. Especially for Australians, the experience of combining lectures with stepping out and soaking up the Parisian culture was totally unique. What can I say – the café's, the architecture and monuments, the Champs Elysees, patisseries, champagne, the Eiffel Tower – it all made for a special Congress.

As usual for these conferences, there was a broad range of presentations each day to choose from, and making that choice before the day began was always a good idea. It was apparently the largest Congress held yet, and it seemed believable: it was easy not to see your colleagues for days at a time. Breaks and lunch periods could be spent socialising and meeting new people, and there was ample time to wander through the Exhibition halls, or to view the poster presentations. It is a great forum for providing a deeper understanding of bleeding disorders and their management, whether from the viewpoint of a person living with a bleeding disorder or of a health professional.

NURSE'S WORKSHOP

For the different health professions, separate workshops were held the day before the start of the Congress. These provide a rare opportunity for professions from the same field around the world to sit in a room together and discuss issues that affect each group similarly. Amongst other things, the nurses considered topics such as joint examinations, nursing research, and heard of planned projects for written resources funded by WFH.

PERSONALISED PROPHYLAXIS

- Peter Collins

One presentation at the Congress that gained my interest (amongst many) was by Dr Peter Collins (Cardiff, UK), who discussed the usefulness of personalising a prophylaxis regime. It is well known that prophylaxis hinders the natural process of haemophilia-related joint disease, and so one of the major conclusions of past studies has been to start prophylaxis early to prevent joint disease. Keeping factor trough levels above 1% for as long a period as possible is one rationale for prophylaxis, and this is often achieved by using a simple weight-based regimen.

An alternative is to tailor the prophylaxis according to the individual's needs at the time, using pharmaco-kinetic (PK) studies (or half-life studies), and collaboration between the individual, his family and the Haemophilia Treatment Centre. Prophylaxis regimens differ for toddlers, teens, young adults and older adults, and as an individual's circumstances change over time, a prophylaxis regime should change to reflect this.

Therefore it makes sense that the dosage of prophylaxis, or the frequency, or the timing of administration, will differ over time. A prophylaxis regimen of three times per week may be a common prescription, but may not be the best one for the individual at a particular time, and PK studies may reveal that individually tailored doses given at different time points may be more appropriate to suit the person's current needs. Something perhaps worth considering!

So, all in all, a great conference that was well worth attending, and we look forward to hosting the next congress on home turf for the first time, in Melbourne, 2014.

Auburn McIntyre is Co-Chair of the Australian And New Zealand Physiotherapy Haemophilia Group

A PHYSIO PERSPECTIVE

Auburn McIntyre

Four Australian Physiotherapists, one from each of the Brisbane and Melbourne adult Haemophilia Centres and one each from the paediatric Centres in Adelaide and Melbourne, were extremely fortunate to attend the WFH World Congress in Paris. It was fantastic to meet and exchange ideas with therapists from America, Canada, England, Ireland and many other Centres to exchange up-to-the-minute ideas.

So what did I see and learn in the first two days? There were talks on the past and present 50 years of treatment by Brenda Buzzard, a Physiotherapist from the UK. She talked of resting in bed after a bleed for months and the strict taboo of exercise for people with haemophilia and how such people were often illiterate and unable to work. I wonder how many of the older Australians reading this could share some of their stories?

Anne Jackson, Amanda Hoppenbrouwers and Auburn McIntyre wearing the Melbourne WFH 2014 Congress t-shirt

SPORT

There were lectures on taping and RICE (Rest, Ice, Compression and Elevation) or PRICE (Protection, Rest, Ice, Compression and Elevation). Many talks focused on sports and the importance of careful, considered participation, especially the Germans who have a "fit for Life" program. One talk presented on the reduction in co-ordination and aerobic fitness in people with haemophilia and showed pictures of a successful live-in "Boot camp"

WOUND HEALING

A morning session included a talk on wound healing. Whilst the studies were reported from tests in mice, there was a lot of discussion that whilst a surface wound may heal quickly that in the deeper wound bed, injured blood vessels can take several weeks to heal fully and that during this period it is important not to stress the new blood vessels. It was thought early return to activity in this period may lead to micro bleeds and reoccurrence of swelling.

OTHER HIGHLIGHTS

Other impressive talks included:

- Ankle surgery in Russia where the results seemed amazing.
- Compartment syndrome: where pressure builds up after a bleed, particularly in people with inhibitors, and the urgency of recognising early warning signs such as pins and needles, reduced sensation and loss of movement after an injury. This was seen as critical to obtaining a good result by presenting ASAP to an emergency medical centre.
- The importance of skeletally immature joints being most susceptible to blood induced damage and the current day management of prevention of bleeding and of unloading the joints after a bleed.
- Special taping that may assist pain removal and improve joint and muscle function.

As for the poster display there were many:

- Smart phone apps for home treatment monitoring
- Nintendo Wii for "exergaming" in Rehabilitation
- A poster on "Unintentional skipping": when young people with haemophilia between the ages 13 to 25 were responsible for their own infusions, this UK group reported that there was a 3.5 times increase in the likelihood some people would unintentionally forget prophylaxis.

In summary, an international conference allowed me to see, meet, greet and do so much. So, save the date for WFH Congress Melbourne 2014! You will only regret it if you don't.

Leonie Mudge is the Social Worker at the Haemophilia Centre, Royal Prince Alfred Hospital Sydney

IMPRESSIONS FROM A PSYCHOSOCIAL PERSPECTIVE

Leonie Mudge

What a thrill to be a part of the 2012 World Congress! There were many interesting presentations, and what follows is just a quick report on some impressions.

CLOSE THE GAP

Mark Skinner, outgoing WFH president gave an inspirational opening plenary address announcing a new initiative, the Cornerstone Program, which will particularly target countries and regions where the gap in care for the bleeding disorders communities is the greatest. The program will focus on improving diagnostic capacity, providing basic training in management of bleeding disorders, and strengthening patient organisations. He challenged pharmaceutical companies to make their treatment products more affordable in emerging countries. Jan Willem Andre de La Porte made a staggering donation of half a million dollars to the Close the Gap Program, and offered to match donations $1 for $1 – for more information, go online at www.wfh.org/closethegap .

HEPATITIS C

The second plenary was about new approaches/horizons about the management of hepatitis C. We heard about non-invasive ways of measuring liver fibrosis stages, and new triple therapy regimes coming up. We were encouraged to realise our treatment available in Australia is at the forefront of that available.

PSYCHOSOCIAL WORKSHOP

The day before the Congress opened officially the psychosocial workers attending were able to have a special, smaller meeting to focus on topics of particular interest. There was a talk about working with new immigrants who may be unfamiliar with our cultural system. We were challenged to use interpreters where appropriate and to arrange translations of materials to ensure there was good understanding of the principles of care.

There were interesting discussions on motivational tools to help patients follow the best treatment plan for them. The importance of respectful interactions which incorporate patient's goals, values and lifestyle was emphasized. There was a great discussion about the importance of involving partners and considering their point of view.

Woet Granotten from the Netherlands spoke about the value of talking with our patients about sexuality. Just in case we were shy, we had the chance to practice. Woet popped up later in the Congress, and it was very refreshing to have the topic of sexuality discussed so openly.

An interesting presentation for me was one on mobility aids – in Helsinki during their long, cold winter. Apparently falls are a huge problem in the icy conditions, which of course present a particular challenge for older people with haemophilic arthropathy.

HFA EXHIBITION STAND

The HFA Booth was an absolute dynamo of enthusiasm. Passersby were encouraged to spend a holiday in Australia in 2014, to coincide with the next World Congress – in Melbourne. It was wonderful to see so many from Australia had been able to make the trip. And there were great presentations by some Aussies also.

And finally a tip from an Auckland Haematologist – focus on all your good genes, and play to your strengths, and try to have a sense of humour.

Sharon Hawkins is Haemophilia Counsellor at the Haemophilia Centre at the Royal Perth Hospital

BOYS AND GIRLS JUST WANT TO HAVE FUN! LESSONS LEARNED FROM THE WORLD CONGRESS

Sharon Hawkins

I feel very privileged to have been funded by HFA to attend the 2012 World Congress in Paris and am so grateful for such a wonderful experience. This was my first World Congress and to say I was in awe of the scale of such an event is an understatement.

On the first day attending the Psychosocial Workshop, I was struck by the enormity of the Palais des Congres de Paris, the Congress venue, along with the ability of its expansive Grand Amphitheatre to house the 5400 delegates. The Congress appeared to be impressively well organised and coordinated with signage that left you in no doubt how to navigate your way around.

PSYCHOSOCIAL WORKSHOP

The Psychosocial Workshop began with an Ice Breaker exercise, of course! This was an interesting experience in itself, with a varying array of social workers, psychologists, psychiatrists, outreach workers and some volunteer psychosocial workers and peer supporters all vying for an opportunity to voice some of the challenges and the positive experiences of their work with people with bleeding disorders. What I noticed most from this exercise was the passion everyone brought to their work and the rewards they feel they gain in working with people with bleeding disorders.

Dealing with sexuality and intimacy in people with haemophilia

- Woet Gianotten, The Netherlands

I felt the highlight of the Psychosocial Workshop was a presentation by Dr. Woet Gianotten, a Sexologist from The Netherlands. What started out as some uncomfortable giggles, became rapturous laughter with Dr. Gianotten's quite explicit and hilarious presentation on engaging people with bleeding disorders in discussing sexuality. The message mostly garnered from this presentation, for me, was the use of humour and honesty to get around the health professional's own discomfort in discussing sexuality. Dr. Gianotten talked about the importance of sex providing muscle relaxation, increased oxytocin (commonly referred to as the "love hormone"), less depression and better mood, more orgasm – less risk of prostate cancer and less cardiovascular death and he explained that there are indications that sex has neuroprotective elements even suggesting that bleeds may repair faster. He then talked about the important role of haemophilia health professionals using best practice models which include assessment of sexuality and ensuring we are armed with information about sexuality and strategies to address some of the very normal difficulties people may encounter eg, fatigue, tired muscles, pain, lessened desire, lessened erection and medications which may all affect sexual function.

Implementing musculoskeletal outcome assessments in clinical practice

- Brian Feldman, Canada

Utilising models of assessment was also later discussed in the Congress with a plenary session on musculoskeletal outcome assessments by Brian Feldman from Canada. Successful outcomes of haemophilia care are often measured by the prevention of arthropathy and improved quality of life. Therefore, measuring physical functioning and quality of life are vital to ensuring the ongoing validation of the provision of haemophilia services and the wellbeing of people with bleeding disorders. Some of the assessment tools mentioned are on the World Federation of Haemophilia website and health professionals were encouraged to utilise them in their regular clinical practice. The content of these presentations will add to my practice as a Haemophilia Counsellor and reinforced the necessity of measuring outcomes in psychosocial care.

As the Congress got underway, I found trying to decide which session to attend was made very difficult by the diversity of interesting presentations being offered. I met with my colleagues from Australia, Maureen Spilsbury and Leonie Mudge, and we decided to split the Psychosocial sessions between us so we could attend as many as possible and share the information later.

YOUTH – PREPARING FUTURE GENERATIONS

Chair: Dorothée Pradines, France

Deon York, New Zealand; David Pouliot, Canada; Diego Gavidia Huanay, Peru; Dorothée Pradines, France

I chose to attend a number of sessions on peer support as I strongly believe that the involvement of people in their peer support organisations provides them with the benefit of many people's experiences living with a bleeding disorder and can be the most effective strategy to assist a family with a newly diagnosed child to accept and adapt to the diagnosis. The presentations from patient organisations provided great insight into the services that are provided around the world. In particular I enjoyed the enthusiasm of the presenters in "Youth: Preparing future generations".

Dorothée Pradines, a young woman with severe haemophilia from France, discussed her efforts to establish youth programs and her frustrations that she and her sister appeared to be the only young people prepared to be involved in their National Member Organisation (NMO). Dorothée spoke of her frequent attempts to engage with young people with bleeding disorders through youth activities and events and through social media, mostly which she described as being unsuccessful.

The speakers prior to Dorothee's presentation, Deon York from New Zealand, David Pouliot from Canada and Diego Gavidia Huanay from Peru had all presented the successes they had experienced in developing youth involvement and the strategies they had used. Some of the strategies were to keep activities and events fun, to have ongoing communication through social media, a youth column in the newsletter, a youth page on the website, encouraging leadership and training and have a youth place on the board of the NMO. MAKE IT FUN and the youth will get involved – appeared to be the message of the session. I was inspired by the encouragement that ensued from the other speakers and members of the audience in response to Dorothee's disenchantment at not having succeeded with her goals for youth representation. Dorothee was encouraged to view her attempts as successful, particularly as she had managed to get a handful of young people to the events she'd organised and that in itself is a success.

THE ROLE OF HAEMOPHILIA ORGANISATIONS

Some of the most interesting and inspiring stories I heard from the Congress were the personal stories of people coping with the complexities of living with a bleeding disorder in a developing country with little access to replacement factor. The holistic services being provided by NMOs addressing emotional, psychological, educational, physical and spiritual aspects of life assist people to cope with their bleeding disorder. These presentations reinforced my belief in the powerful role NMOs can have in improving people's quality of life.

COPING STRATEGIES

Chair: Susan Cutter, USA

Susan Cutter, USA; Silvina Grana, Argentina; Lara Oyesiku, UK

Another session I attended was titled "Coping Strategies". The first speaker, Susan Cutter, a Social Worker from the Hospital of the University of Pennsylvania, presented on "Coping with Transitions in Adulthood" and discussed the usual transitions young people go through into adulthood and some of the differences that may be encountered for a person with a bleeding disorder. She noted that some transitions can be stressful but can also be positive aspects of life, like marriage and giving birth.

Susan stated that how a person coped in the past will inform how they cope in the future. This is particularly relevant for people with chronic health conditions. A person's perception of their bleeding disorder can have a positive or negative impact on their transitions through adulthood. Susan indicated that people with bleeding disorders can sometimes experience transitions out of sequence to the norm, for example disability and joint disease, which may occur earlier than the general population, affecting ability to work and increasing the possibility of negative financial impacts. Susan also highlighted people with a bleeding disorder may learn coping skills earlier than their peers which can have a positive impact.

Susan suggested that psychosocial workers assist families repair a balance by strengthening resilience through fostering self efficacy, social supports, stress management, flexibility, hardiness, optimism and helping them develop an internal locus of control. Susan indicated that it is important for people to be able to understand the process of grieving their losses but also to embrace the new. Psychosocial workers can assist people with their perceptions of the challenges and transitions in life, by reinforcing the benefits of change and that change is a normal part of life which can stimulate growth.

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Silvina Grana, Psychologist from Argentina then spoke on the importance and benefit of play as a means of working through the crisis which is often experienced when a child is diagnosed with a bleeding disorder. Silvina talked about playing as being a basic way of life for children, how they learn to socialise and is universal. In Silvina's practice she utilises playing as a means of connecting with children with bleeding disorders and also advocates the use of playing inside the family to improve self knowledge, self esteem and relationships within the family. Silvina uses play to assist children and their families to resolve conflict and cope with traumatic situations. Silvina has integrated playing workshops into her practice and has produced a range of books for children with bleeding disorders.

Another speaker, Lara Oyesiku, a nurse from Oxford Haemophilia Treatment Centre also encouraged the use of play to improve a child and their family's knowledge base and psychological wellbeing. Lara discussed using a play specialist and encouraging medical staff to play with children undergoing treatment as a means of distracting and of educating through role playing infusions with a teddy bear. Lara has observed that children find it easier to talk while playing and can express their thoughts more readily. Lara also noted that the risk that children are disturbed by having to attend hospital can be reduced by suitable preparation by parents and staff. Children attending the Oxford Haemophilia Treatment Centre are given a play box to get them ready for prophylaxis. The play box includes treatment supplies, like syringes, swabs, bandaids etc., which the children can play with and become familiar with. Lara indicated that they had experienced good outcomes for children that are well prepared.

I was surprised to realise how little I incorporate play into my interactions with children with bleeding disorders and have been inspired by all these presentations to perhaps lighten up and have a bit more fun!

I've only highlighted a few of the sessions I attended and there were many more sessions at the Congress which I would have liked to been able to see. Hopefully I will have the opportunity to make it to Melbourne in 2014 and again experience the breadth of knowledge and support that was being shared in Paris.

UPDATE ON GENE THERAPY FOR SEVERE HAEMOPHILIA B

At the recent World Congress in Paris, Professor Edward Tuddenham from the UK gave an update on the haemophilia B gene therapy clinical trial that is being conducted by a combined UK and USA team.

Prof Tuddenham pointed out that it is over 20 years since the first signs that a type of gene therapy for haemophilia B might be successful. He explained that the "basic aim of gene therapy is to correct a genetic defect by introduction of segment of DNA or RNA into a patient's cells, which makes good the defect", but that "until very recently, no effective gene therapy has been reported in any type of bleeding disorder".

The success of the Anglo-American study has been greeted with great interest worldwide. At the World Congress, Prof Tuddenham reported that seven people with severe haemophilia B had now been treated at three dose levels with the gene therapy using the AAV virus mediated transfer of factor IX. Results on safety and efficacy were:

- Two people treated at the highest dose level developed immune mediated transaminitis (a transient elevation of liver enzymes) which was rapidly controlled with a short course of Prednisolone.
- No acute or long-lasting toxicity has been observed.
- All seven people have achieved a new factor IX baseline level ranging from 2% to 5%.
- All have been able to reduce or eliminate the need for regular factor IX infusion.

The future plan for the clinical trial is to continue treating up to 30 more people at the higher dose level, monitoring for evidence of an immune response, like that seen in the two people in the original trial, and treating that with prednisolone if it occurs. The aim is to refine and improve the gene therapy so that it can be used more widely to treat haemophilia B.

Gavin Finkelstein, HFA President, attended the gene therapy presentation and commented, "It's exciting to see the new developments in gene therapy. It's been a long time coming – it has seemed like it has been a real struggle to move gene therapy from the initial studies to the next phase of clinical development. The progress with gene therapy clinical trials in haemophilia B is very promising for the future – but, that said, we need to know that it will be safe, effective and affordable."

REFERENCE

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GENE THERAPY TRIAL SHOWS PROMISING RESULTS

Mark Brooker

Scientists have made a remarkable breakthrough in the development of gene transfer treatment for haemophilia. A team of researchers based in London, led by doctors Amit Nathwani and Edward Tuddenham, published unequivocal evidence of success of this technology in the treatment of haemophilia B.[1] An early abstract, hinting at success in the study, was first reported at the WFH 2010 World Congress.

A total of six patients with severe haemophilia B were treated with injections of the adeno-associated virus (AAV) vector carrying the normal factor IX gene. All six patients showed therapeutic response to the factor IX gene administration because post-injection they showed higher measurable clotting factor levels and required either less clotting factor concentrate or none at all in their daily lives. One patient receiving the highest dose of the vector has maintained factor levels between 8 per cent and 12 per cent for beyond 6 months. The study is ongoing.

We have known for years that treating haemophilia through gene transfer is possible because it has been demonstrated to work long-term in animals, including mice and dogs. In dogs with haemophilia, therapeutic levels of clotting factor production have been achieved for more than eight years after a single gene transfer administration. The challenge has been in reproducing that success in humans.

The technique employed in this study was simple, with the normal factor IX gene being delivered into a peripheral vein in a tiny carrier called a vector. These are small viruses that infect humans and some other primate species but do not cause any disease. There are different types of AAV with attraction to different human tissues, such as the liver. The strategy is that the modified AAV with the factor IX gene inside it is injected into the vein of a patient and travels to the liver where it delivers the normal factor IX gene into liver cells. These liver cells then begin to produce factor IX and the patient's factor level rises.

Previous attempts to use AAV vectors in humans with haemophilia B did show some success in raising factor levels. In these early trials, researchers used the AAV2 virus particles in very large numbers to overcome inefficiency in entering liver cells. However, the response was short-lived (four to six weeks) and factor production in the liver eventually stopped as the human immune system destroyed the liver cells that contained the virus. Furthermore, as most humans have been previously exposed to this form of the virus and therefore carry antibodies to it, they cannot be treated effectively with this vector.

This most recent trial is exciting because it used a redesigned AAV8 vector that has greater efficiency in entering liver cells and requires lower doses which avoids the major immune response seen in earlier trials. Furthermore, knowing that an immune response to the vector could be a problem, the patients in this study were carefully monitored. When two of them showed signs that their bodies were attacking the vector, they were given a short course of steroids to suppress this immune response and their factor levels remained elevated.

Gene therapy for haemophilia B will move forward in a process much like that for the development and approval of a new clotting factor concentrate. The six patients from this trial will continue to be monitored. A further clinical trial, under the supervision of regulatory authorities in the U.S.A. and Europe, will be conducted to show the safety and efficacy of the treatment and to determine the optimal dose. If that trial is successful, the regulators would then approve it for use in patients.

The success in treating haemophilia B suggests that haemophilia A could also be treated using this strategy. However, the factor VIII gene is much larger than the factor IX gene and it remains to be seen if the vector used for haemophilia B gene therapy could carry the larger factor VIII gene. It may be that a different and larger vector is necessary to effectively deliver the factor VIII gene. In addition, the immune response to factor VIII (factor VIII inhibitor development) is also a potential challenge for factor VIII gene transfer studies.

The patients who volunteered for this and previous gene therapy trials for haemophilia have made an incredible contribution in the advance towards a cure for haemophilia. The WFH has been an early supporter of gene therapy, including this trial. "We support clinicians and researchers working in the field to achieve this goal," said WFH president Mark W. Skinner. "We are most grateful to the researchers who pioneer these studies and to the patients who have participated in clinical trials."

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Editor's note: "hemophilia" has been altered to "haemophilia" in this article to reflect Australian spelling

This article is abridged from the article published on the Hepatitis Australia web site www.hepatitisaustralia.com in February 2012 and is reprinted with permission

A GUIDE TO CURRENT AND EMERGING HEPATITIS C TREATMENTS

The way that hepatitis C is treated in Australia is going through a period of rapid change due to significant research breakthroughs in our understanding of the virus, and the development of different types of drugs that can be used to treat the infection.

CURRENT HEPATITIS C TREATMENT

The current treatment for hepatitis C within Australia has not changed significantly since 2003 when a regimen of weekly pegylated interferon injections and twice daily ribavirin tablets was introduced as the standard treatment.

Response to treatment

The genotype or strain of the virus that a person has is a major predictor of response to treatment. Genotype 1 is harder to treat and typically requires 48 weeks of treatment. In comparison, the easier to treat strains, genotypes 2 and 3, typically only require 24 weeks of treatment.

The person's response to treatment is monitored through blood tests taken at intervals during treatment to measure the amount of virus present in the blood. If a person does not respond well during the earlier stages of the treatment regimen it means that a successful outcome is not achievable and the treatment is therefore ceased. Varied treatment outcomes are possible, the main ones being:

- Null response: the amount of virus does not drop significantly
- Partial response: the amount of virus drops significantly, but never reaches undetectable levels
- Relapse: the amount of virus drops to undetectable levels while on treatment, but becomes detectable again within six months of completing treatment
- Sustained Virological Response (SVR): the amount of virus drops to undetectable levels while on treatment and remains undetectable when tested six months after the end of treatment.

It is important to note that when clinicians or others talk about a 'cure', they are referring to an SVR. Typically, a cure is achieved following pegylated interferon and ribavirin treatment in around 50% of people with genotype 1 and 70-80% of people with genotype 2 and 3.

Recently, a new genetic test has been developed called 'IL28B' which offers a strong pre-treatment prediction of treatment response for people with genotype 1. The individual's genetic make-up is tested and people fall into one of three groups – CC, CT or TT. Those within the CC group are most likely to achieve a cure. Those in the CT and TT groups have a lower chance of achieving a cure with the current treatments. The test has obvious advantages in helping to guide an individual's treatment decision, however the drawbacks should also be considered prior to requesting or consenting to have the test. Privacy concerns, potential use of the results by insurance companies, the impact of receiving an unfavourable result and implications for other family members should all be considered prior to testing. The cost of the test is not subsidised and charges can vary between pathology services.

While genotype and IL28B grouping for people with genotype 1 can help predict the likely treatment outcome, other factors also play a part. For example, we know that people with lower levels of liver disease, pre-menopausal women and those in a healthy weight range all have a better response to treatment than those with significant liver disease, men, post-menopausal women and people who are over-weight. Knowing what can affect treatment outcomes helps people to prepare for treatment and may also influence the timing of treatment to optimise outcomes.

Timing of treatment

For many people, the timing of treatment is important and the arduous process can be made a little easier if people are well prepared, have good social supports and an understanding employer who will provide flexibility in hours of work. Some women may be keen to try treatment prior to starting a family due to their concerns around the 3-5% risk of transmitting the hepatitis C virus to their baby. For others, knowing the stage of their liver disease is a key factor in deciding the best time for treatment. Those with lower levels of disease might choose to postpone treatment until they are better prepared. This however, has to be balanced against the knowledge that higher cure rates are achieved in the earlier stages of liver disease.

Fibroscan™

A relatively new test called a 'Fibroscan™' is now in use to establish the stage of liver disease by measuring the level of liver scarring. It is a quick, simple and non-invasive test similar to an ultra-sound test. Access to a Fibroscan™ test is improving, however, it is not available everywhere. The Fibroscan™ test is much preferred by most people with chronic hepatitis C to an alternative test called 'liver biopsy' which involves taking a sample of liver tissue with a

needle. However, a liver biopsy may be recommended by specialists in some circumstances.

OVERVIEW OF EMERGING HEPATITIS C TREATMENTS

Research into new hepatitis C treatments has resulted in major new classes of drugs being tested in clinical trials. The rapid evolution in the treatment of hepatitis C is expected to result in significantly improved cure rates and potentially reduced duration of treatment making it a much more acceptable option for many.

It is anticipated that the new drugs will become available in Australia in three sequential waves:

Wave One

Treatment with pegylated interferon and ribavirin will continue unchanged for people with easier to treat strains of the virus such as genotype 2 and 3, however, an extra drug from a class of drugs called Direct Acting Antivirals (DAAs) will be added to treatment for people with genotype 1. It is expected that the first two drugs from this class to become available in Australia will be boceprevir or telaprevir. These two drugs work by blocking an enzyme called 'protease' which is needed by the hepatitis C virus to multiply; they are therefore known as 'protease inhibitors'. The addition of either of these drugs to standard treatment will improve cure rates significantly and, for some people, will also reduce time on treatment. However, further side-effects are experienced.

Wave Two

Treatment with pegylated Interferon and ribavirin will continue as the mainstay of treatment for all genotypes, but additional new drugs will be added, not only for genotype 1, but also for other common genotypes, such as genotype 2 and 3. The new combinations may see the addition of one or two DAAs, for example protease inhibitors, polymerase inhibitors and NS5A inhibitors. These drugs act in different ways to block the ability of the hepatitis C virus to multiply. It is anticipated that these new drugs will result in improved cure rates for all genotypes and reduce the time on treatment for some people. Treatment is expected to become more tolerable over time as the frequency and number of tablets needed is reduced.

Wave Three

During wave three, treatments will no longer include pegylated interferon, but it is thought they will still incorporate ribavirin. Various DAAs will be used in combination.

Currently there are numerous clinical trials underway to test the effectiveness and safety of various combinations of DAAs for different genotypes. These new treatments show great promise for very high cure rates, shorter time on treatment and much reduced side-effects compared to current treatment regimens.

Availability of the new treatments in Australia

While there is great excitement surrounding the potential to move to non-interferon treatments for hepatitis C, it could take 5 years or more for these to become available in Australia. In the meantime, it is expected that other new drugs will become available which will offer considerable advantages over current treatment.

The first wave of new drugs, boceprevir and telaprevir, are currently in the process of registration with the Therapeutic Goods Administration, assessment by the Pharmaceutical Benefit Advisory Committee and approval by the Federal Government.

It is hoped that this process will be completed before the end of 2012. Once approval processes are completed they will be listed on the Pharmaceutical Benefits System (PBS) which means the Federal Government will subsidise the cost of the medication making it accessible to Australians who meet the eligibility criteria.

The first wave of new treatments, adding boceprevir and telaprevir to treatment for people with genotype 1, is anticipated to continue for several years until the next group of DAAs described in the second wave complete the clinical trial process and gain PBS listing. Optimistically, another 2 to 3 years after that the non-Interferon treatment described in wave three will start to become available in Australia.

It is important to note that it is very difficult to predict with any degree of accuracy when new hepatitis C treatments will be listed on the PBS. Regardless of how promising any clinical trial is in the initial stages there are many hurdles to overcome and not all promising new drugs manage to complete the clinical trial process. For those that do, the subsequent rigorous TGA, PBAC and Cabinet assessment and approval process also takes a considerable period of time and is not predictable. Typically the availability of new treatments in Australia lags well behind the USA.

Realising the potential of the emerging treatments

The new treatments becoming available in Australia have great potential to offer the chance of a cure for many people living with chronic hepatitis C. If you are currently living with chronic hepatitis C this therefore is a good time to reconsider your treatment options.

For further information visit the Hepatitis Australia website www.hepatitisaustralia.com

Wendy Poulsen is Haemophilia Physiotherapist at Royal Children's Hospital, Brisbane.
Auburn McIntyre is Haemophilia Physiotherapist at Women & Children's Hospital, Adelaide

HAVE YOU SEEN A PHYSIOTHERAPIST RECENTLY?

Wendy Poulsen and Auburn McIntyre

Physiotherapists at Haemophilia Centres in the developed world aim to screen people with severe haemophilia yearly. This is in addition to treating acute bleeding episodes and other musculoskeletal problems. People with moderate and mild haemophilia, in general, are screened each two years. The most common assessment is the Haemophilia Joint Health Score which reviews the elbow, knee and ankle joints to assess changes. The screens are done because:

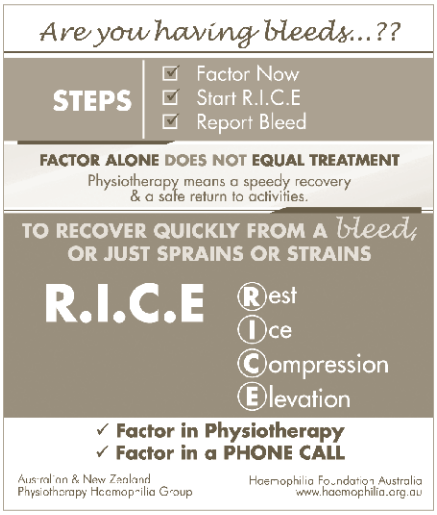
- Joint and soft tissue bleeding episodes experienced by individuals with bleeding disorders have the potential to result in chronic musculoskeletal and movement dysfunction
- Due to the physiotherapist's specialized training in evaluation and treatment of movement dysfunction they are a valuable part of a comprehensive team. At routine clinic visits they can identify any new musculo–skeletal issues and make appropriate recommendations
- The physiotherapist is able to instruct the patient and their family in a comprehensive home care program to address individual needs, particularly following a bleed or injury. They can advise on early detection of a bleed; provide knowledge on how to treat and manage a bleed following factor replacement and instruction on immobilization or splinting. Physiotherapists can also provide progression of exercises without the risk of causing further bleeding, as well as specific exercises for the individual problem areas.

The major role in the treatment of haemophilia is to maintain, or restore muscle and joint status, thus minimising the potentially disastrous effects that result from repeated bleeds.

Physiotherapists also recognise changes related to aging, for example, loss of muscle strength, endurance and reduced balance. These factors may heighten people who have haemophilia at an increased risk of falling.

Physiotherapists can:

- Improve muscle strength and co-ordination
- Reduce pain
- Enhance balance skills
- Prevent or reduce complications from bleeds
- Assist in early resolution of a bleed
- Offer advice in the prevention of further injury to susceptible joints /muscles
- Promote safe involvement in fitness and sports participation
- Educate and stimulate people with haemophilia their families and friends to take an active role in their care
- Provide advice for their chronic disease management
- Assist with management of normal musculoskeletal issues
- Provide of splinting, orthotics and assistive devices for optimal function.



SO

If you have severe haemophilia and in the last year haven't seen a physiotherapist who has experience with haemophilia

If you have moderate or mild haemophilia and in the last 2 years haven't seen a physiotherapist

If you have had a recent bleed or new aches and pains that you are not sure about IT'S TIME……

Call your physiotherapist at your Haemophilia Treatment Centre!

This article has been adapted from an article published in *Bloodline*, June 2012, the newsletter of Haemophilia Foundation of New Zealand, and is reprinted with permission. Linda Dockrill is the Southern Outreach Worker at Haemophilia Foundation of New Zealand

HOW TO GET THE MOST OUT OF YOUR CLINIC VISIT

Linda Dockrill

If you or your child has haemophilia, von Willebrand disorder or another clotting disorder, you will have regular clinic appointments with the Haematologist and/or Paediatrician. These may be six monthly, yearly or less frequently depending on your situation.

Clinicians who have chosen to work with people with bleeding disorders have medical training, knowledge and experience in this area, making them experts in their field. As the bleeding disorder community is small, and these are lifelong disorders, you often get to know each other very well. This can be an advantage for everyone, as there is time to build relationships and learn about how to work together more effectively.

While getting along well with clinical staff is a bonus, they don't need to be our friends for them to do their jobs effectively. People with bleeding disorders need clinical staff to be knowledgeable, caring, attentive and good at explaining things in a way you can understand it. It is their responsibility to ensure you have all your questions answered and you understand what plans have been put in place.

YOUR RESPONSIBILITY: BE PREPARED

It is your responsibility to come to the clinic appointment on time and prepared so that you can make the most of your time with them.

If you feel lacking in confidence or intimidated by the hospital setting, take a support person with you. This can be a family member or friend. Your Haemophilia Social Worker or Counsellor would be happy to attend clinic appointments with you.

Before your appointment, ensure that you have been keeping your treatment diary up to date and have recorded bleeds, factor administration and any symptoms or issues that have been occurring. Write down the questions you would like to ask about treatment or the bleeding episodes. Keep note of any additional medications that have been prescribed by your GP or any other specialist since the last clinic visit. Are they causing any side effects that you should mention?

NEGOTIATE A TREATMENT PLAN

Work with your Haematologist, Paediatrician, Haemophilia Nurse or Physiotherapist to negotiate a treatment plan that is required to treat your bleeding disorder but is also realistic. Be honest with the clinical staff about your desire to exercise, the likelihood of making it to the gym, which physio exercises are painful to do and how the treatment plan will work in with your lifestyle. While there are ideals to administering factor, if you are able to be honest about the difficulties, clinical staff can assist you with problem solving around this. If you are a parent of a young child who suffers from needle anxiety, explain your concerns and ask for assistance with this. If you are struggling with early morning treatments, as many parents of young children do, tell clinical staff this so that they can provide you with medical information about this. Negotiating a treatment plan that will meet both of your needs is important and more likely to be followed over time.

REFERRALS

If a clinician discusses making a referral to another specialist, ask what the time frame is for hearing a result. It may help to note the possible date in your diary or calendar. Some tests take days, others take many weeks, so make sure you get an idea of how long it is likely to be until you hear any news as waiting for test results can be stressful. If you don't hear anything back in the time frame you were given, speak to your Haematologist or Haemophilia Nurse or ask your Haemophilia Social Worker or Counsellor to follow up on your behalf.

ASK FOR ANSWERS TO YOUR QUESTIONS

At the conclusion of your clinic appointment ask the clinician to summarise what you have discussed together and clarify any areas of confusion. Ensure you know the answer to the following questions:

- Where do we go from here?
- If this doesn't work what do I do?
- What should I expect to happen?
- Who do I call if there is a problem?

IN SHORT

Getting the most out of your clinic appointment depends a lot on you taking responsibility for being prepared, bringing along someone to support you or to write down what you discuss, agreeing to a plan, asking all your questions and being honest about what will work for you once you walk out the door. If you need further support with getting the most out of your clinic visits, speak to your Haemophilia Social Worker or Counsellor.

"Preparation is the key to success" *Alexander Graham Bell*

Suzanne O'Callaghan is Policy Research and Education Manager, Haemophilia Foundation Australia

HEPATITIS C UPDATE

Suzanne O'Callaghan

In July 2012 the Pharmaceutical Benefits Advisory Committee (PBAC) recommended that the two new protease inhibitors, telaprevir and boceprevir, should be included in the Pharmaceutical Benefits Scheme (PBS) for the treatment of hepatitis C. The next step is for the new therapies to go to Federal Government for Cabinet assessment and, potentially, approval. If they are approved, they will receive a government subsidy which will make the treatments more affordable to people undergoing treatment.

NEW APPROACHES TO HEPATITIS C

If these protease inhibitors are approved, it will be the first step in the availability of the new classes of hepatitis C drugs. The treatment and care landscape for hepatitis C is evolving rapidly and this is of great relevance to the people with bleeding disorders who live with hepatitis C. At the 2012 WFH World Congress, Professor Fabien Zoulim from France highlighted that people with bleeding disorders who currently have hepatitis C and acquired it through their treatment products have had chronic infection for more than 20 years and many are starting to experience the problems of advancing liver disease.

Prof Zoulim made a number of points that are of particular interest to people with bleeding disorders:

- Non-invasive tests such as Fibroscan® have been a successful alternative to liver biopsy in assessing hepatitis C-related liver fibrosis
- The treatment of people with bleeding disorders for hepatitis C is no different to that of other people with hepatitis C
- If a person is seeking treatment with one of the new triple therapy combinations that includes telaprevir or boceprevir and has previously had unsuccessful treatment, it is important to understand why the previous treatment failed to assess the likelihood of success with the triple therapy (if you fall into this category, discuss it with your hepatitis specialist)
- The potential for side-effects with these new triple therapies, particularly anaemia and rash, needs careful monitoring and management by the hepatitis medical team
- It looks hopeful that a combination of direct acting antivirals (DAA) will provide safe and effective interferon-free therapies with shorter treatment courses and will become available in the near future
- With these DAA combination treatments, issues relating to drug resistance will need to be overcome; potential drug-to-drug interactions will also need to be monitored, particularly for people who are co-infected with HIV and being treated with HIV antiviral medications
- However, the clinical development of the new DAA combination treatments is likely to be more rapid because the treatment courses are shorter.

REFERENCES

F. Zoulim and F. Bailly. New approaches to the management of hepatitis C in haemophilia in 2012. Haemophilia 2012;18(Suppl. 4):28-33.

F. Zoulim. New approaches/horizons to the management of hepatitis C in haemophilia in 2012. Presentation at the World Federation of Hemophilia World Congress, Paris, France, 8-12 July 2012.

WORLD HEPATITIS DAY

World Hepatitis Day was celebrated globally on 28 July 2012.

With a theme of ***"Love Your Liver on World Hepatitis Day"***, the national campaign aimed to raise awareness of liver health with the general community and present viral hepatitis B and C neutrally as health conditions linked to liver health. The intention of this is to normalise viral hepatitis and reduce stigma.

Around Australia there was a variety of awareness-raising events and activities. Events ranged from informative workshops and forums, to art exhibitions, information stalls and Love your Liver events.

Hepatitis NSW, Hepatitis VIC and Hepatitis SA all took part in the global "Three Wise Monkeys" Guinness Book of Records attempt. The three wise monkeys theme ("see no evil, hear no evil, speak no evil") was used to highlight that hepatitis is being ignored around the world.

The Olympics theme of the national campaign poster, with the tagline ***"Get your liver over the line"***, was developed specifically for the timing of World Hepatitis Day, which occurred on the first day of the London Olympics.

As a Partner in the national World Hepatitis Day Campaign, HFA worked with Hepatitis Australia on the annual national awareness campaign and is committed to reducing hepatitis C stigma and discrimination and improving liver health in Australia. HFA and State and Territory Foundations published articles in their newsletters and HFA put up a window display for the people who pass by the office in High St.

Sharon Caris is Executive Director,
Haemophilia Foundation Australia

SOUTH AUSTRALIA

Sharon Caris

Haemophilia Foundation Australia (HFA) keeps in touch with many friends and former members of Haemophilia Foundation South Australia (HFSA) in various ways, and anyone is encouraged to contact HFA staff if there are issues that we can help with. Please feel free to join in on some of the national activities such as our upcoming Red Cake Day during Haemophilia Awareness Week.

Although it was necessary for HFSA to voluntarily wind up earlier this year, the HFA Board has taken steps to ensure local issues affecting people with bleeding disorders in SA are brought to the Council table by inviting former HFSA President Paul Bonner to join the HFA Council as an observer. We will continue to encourage South Australians to participate and will try to develop informal processes for South Australians to contribute their ideas.

Paul Bonner has participated in the Haemophilia Treatment Network for several years as a community member, and will continue in this role. Paul and Sharon Caris, HFA Executive Director attended a recent meeting of the Network which includes treating health professionals and health department officials involved in the provision of care and treatment to people with bleeding disorders in South Australia and discussed matters of concern to the community.

Several young people from South Australia have been interested in Factored In, the HFA youth website, and we encourage young people to join factoredin.org.au or contact Kate Walton, Youth Project Officer at HFA to find out more about the Youth Project.

VALE DR JOHN VINER LLOYD OAM

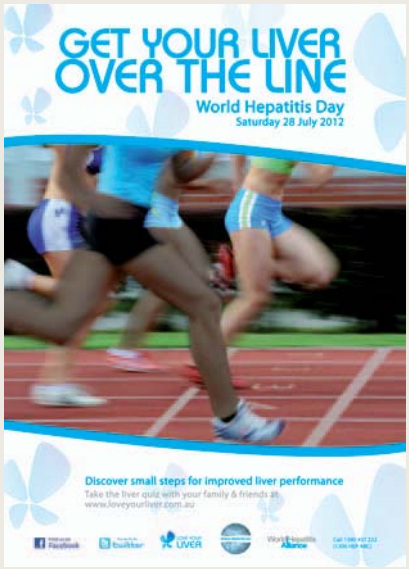
Everyone who knew Dr John Lloyd OAM will have felt a great sadness to learn that he died in August. Dr Lloyd was a greatly respected doctor and scientist who worked for many years at IMVS and the Royal Adelaide Hospital, as well as overseas. He was a great supporter of the bleeding disorders community and was the Chair of the HFA Medical Advisory Panel at the time the group was incorporated as the Australian Haemophilia Centre Director's Organisation (AHCDO). He will be well remembered by his patients and their families for his care and by fellow doctors, students and the many people he worked with during his wonderful career. This was aptly recognised when he was awarded a Medal of the Order of Australia for his service to medicine and the field of haematology, to medical education, and to professional associations in June this year.

See the exciting news about TEAM.FACTOR on the back page of this issue.

The HFA facebook page also became a place to highlight the World Hepatitis Day events around the world – and to issue a challenge to HFA facebook fans to join us in finding out how to be kind to their liver and try out one liver-friendly activity on 28 July. More than 500 people read the HFA facebook post on liver-friendly breakfasts that followed!

Interested in more information about liver health?

- Visit the Lover Your Liver web site (www.loveyourliver.com.au)
- Download liver-friendly recipes from the web site – or add your own!
- Take the liver quiz on the web site.



Kate Walton is Haemophilia Foundation Australia Youth Project Officer

UNDERSTANDING THE NEEDS OF YOUNG PEOPLE WITH BLEEDING DISORDERS

Kate Walton

As part of the Beyond Prophylaxis project, HFA carried out a needs assessment to help understand the needs of young people affected by bleeding disorders in Australia.

CONSULTATION

To understand the issues affecting young people with bleeding disorders, we first looked at the literature in Australia and around the world. After this, we consulted directly with young people in Australia, parents, the wider bleeding disorders community and haemophilia health professionals about what they thought the issues were for young people and what would assist them.

Invitations to take part in the consultation were sent out through HFA and State and Territory Foundation newsletters, the HFA facebook page and Haemophilia Centres.

Thanks to all who participated:

27 young people who have a bleeding disorder, carry the gene, or are siblings

9 parents

7 State/Territory Foundations

16 haemophilia health professionals

The needs assessment report was completed in July 2012. It outlines various issues which were raised in the consultation and includes the topics of information that participants believed need to be targeted at young people to help them better understand their bleeding disorder, for example information about sport, travel and transition presented on a web site or other media source. The report also recommends actions to address these issues and others raised in the consultation process.

RESULTS

Major issues identified in the needs assessment included:

Living a 'normal' life

More effective treatment and care for young Australians with bleeding disorders has created a new generation which has expectations of living a 'normal' life. However, this has meant that young people now have fewer opportunities to connect with their peers or older members of the community through Haemophilia Centre visits or clinics and to talk with others about managing the bleeding disorder-related problems that they nevertheless do experience or the long-term impact of bleeding complications. For young people at all levels of severity, experiencing the reality of living with a bleeding disorder and the complications that can result, when they have been educated to expect a 'normal life', can be isolating and confronting.

Disengagement and non-compliance

Consultation with the community, parents and health professionals has highlighted that that as teenagers, many young people also disengage from the bleeding disorders community and do not comply with their treatment and care regimes for a range of reasons:

- Peer pressure and wanting to be 'normal' or needing to look 'cool'
- Pressures of study
- 'Don't care' attitude
- Maturity issues and stages of development
- That most people with haemophilia are male, at this age they feel "invulnerable", and "boys don't talk".

Perspectives of young people

In contrast, consultation with young people indicated that they experience a range of problems due to their bleeding disorder at this age, including:

- Understanding their medication and how to manage it
- Managing pain
- Missing out on school, sport, travel or work due to bleeding problems or appointments
- Feeling different and alone
- Discrimination, bullying and stigma
- Issues in managing their relationships with health professionals
- Negotiating health care services and understanding what is available
- A need for health services to be more flexible to accommodate their needs.
- It is important to them to get to know their body as it develops during puberty and learn how their bleeding disorder affects them individually.

One spoke of a "hating my haemo" phase and that while he was now resigned to living with his bleeding disorder, he still wished he did not have it.

Younger people in the 13-15 year age group described particular difficulties in learning the skills to:

- Manage their treatment, including self-injection
- Explain their health condition or health needs to others, for example, to friends and other school students, friends' parents, teachers and health professionals.

Risk-taking and experiencing life

For Haemophilia Foundations and health professionals, discontinuing treatment, uninformed decision-making and risk-taking related to career choice, sports and other physical activities was of great concern because of the permanent musculo-skeletal damage or injury that could result. They noted that disabilities acquired through haemophilia or injury could cause young people to drop out of participation in sports, school, work, or with their peers, and could lead to them becoming disempowered and isolated. They were aware of problems with depression, anxiety and self-esteem among young people with bleeding disorders.

Young people, on the other hand, wanted the opportunity to explore their independence and have the opportunity to make mistakes and learn from them.

Environments for connection

Young people, community members and health professionals saw the Beyond Prophylaxis project as an opportunity to take positive steps towards creating spaces where young people could connect, share experiences and learn from each other, inform themselves about bleeding disorders, develop life skills and have some fun together.

They agreed there should be a mix of options for contact and connection:

- Web-based and social-media technology based communication tools
- Face-to-face meetings, such as national youth camps, weekends or "catch-ups"
- Using the telephone and Skype to connect
- Other information resources, including print based.

Peer education and mentoring

It was important to young people to have opportunities to share stories and learn from each other's experiences; for example, to have video and story blogs on a web site and their own examples of 'how to'. They also agreed that it was important to develop a group of older young people who could take on the role of peer educators and mentors, with the potential for being community leaders in the future.

Engaging the disengaged

Engagement with young people with bleeding disorders in the consultation was difficult and highlighted the need for face-to-face connection and other strategies to build trust and confidence in HFA's work in this area. All groups consulted referred to young people with bleeding disorders who are isolated and difficult to engage, often because they prefer not to identify with having a bleeding disorder or with the bleeding disorders community or do not find the community activities attractive. They noted that isolation from peers with bleeding disorders and community could contribute to the sense of being alone and decrease opportunities for learning effective ways to manage their bleeding disorder from peers, community mentors or health professionals. Further investigation into engaging with this group and understanding their needs would be valuable.

Preparing adolescents for independence

Health professionals also noted the need to prepare pre-teens for the skills they will need to become independent in the future, before they start to disengage from their Haemophilia Centre during their early teenage years. This work will need more exploration to enable it to be scoped.

Proposed web site

The project's web site could provide a more attractive forum for young people to discuss their needs and experiences and explore sensitive issues anonymously and honestly. It could provide evidence-based information on priority issues such as health, lifestyle and life skills for working, relationships and socialising in the language of the young people accessing it and create a space for peer education and connection. Ensuring that this is developed appropriately and any forums moderated carefully would involve the work of a skilled professional.

Summary of Recommendations:

- Target and promote the project at young people affected by bleeding disorders (who have a bleeding disorder, carry the gene, or are a sibling) in the 13–30 year age group
- Investigate how to reach young people who are currently disconnected from the community
- Explore the "hating my haemo" phase with young people
- Explore a range of options to enable young people to communicate with each other, eg web site, social media, telephone, Skype and face to face (camps, weekends and "catch-ups")
- Develop a web site that has:
  - Sections to share experiences and personal stories
  - Accurate information in 'youth friendly' language
  - Information for girls and siblings
  - Information on priority issues
- Develop the role of young people as peer educators and mentors
- Provide feedback and education to schools, the wider community, health professionals and government on priority issues for young people
- Investigate how to prepare pre-teens for the skills they will need to become independent.

The needs assessment will be published by HFA in the near future and will be available from HFA, State and Territory Foundations and on the HFA web site – www.haemophilia.org.au.

Contact Kate Walton, HFA Youth Project Officer, if you have any questions about the needs assessment or the project – phone 1800 807 173 or email kwalton@haemophilia.org.au.

Kate Walton is Haemophilia Foundation Australia Youth Project Officer

HFA YOUTH PROJECT UPDATE

Kate Walton

FACTORED IN WEB SITE

The Factored in web site was launched online on Tuesday 26th June 2012 with a launch video that displays the first time someone visits the web site. The video will be available for a couple of months to view on the website.

The web site has many features based on feedback from the weekend workshop and the YWG including:

- Up to date and accurate youth based information
- A young, fun and colourful design
- Ability to 'join' the site and become members
- Make comments on other people's stories
- The question and answer section where anyone can ask a question and have it answered by an expert (you don't have to be a member to ask questions)
- Search function – to find information on the website easily and quickly
- Share function – to share and print pages from the website including the ability to share pages on facebook, twitter, email and many others
- Mobile optimization – the website can be viewed on smart phones.

CURRENT STATISTICS

These statistics cover the period from when the web site was launched (26 June 2012) until the end of August 2012:

The website currently has 50 members, 36 male/14 female

Visits to the site: 801

Unique visits
(a new user each time): 346

Visitors from: Australia, France, Denmark, United States, New Zealand, Ireland, Nepal and Turkey.

PARIS CONGRESS 2012

HFA had a poster about the youth web site accepted for presentation at World Congress in Paris and the abstract was printed in the abstract book. Much positive feedback was received about the poster, and we have received follow up emails from haemophilia organisations around the world asking questions about the project and requesting copies of the poster.

NEXT PHASE

The next phase of the Beyond Prophylaxis project is based on some of the recommendations from the Needs Assessment (see page 26) specifically around face-to- face contact, mentoring and developing a sustainable youth program.

Work on the mentoring program has commenced. This will involve working with young community members to look at ways of communicating and providing support to each other. It will also involve working with State and Territory Haemophilia Foundations and haemophilia health professionals.

CONNECTING YOUTH:

engaging young people with bleeding disorders in a communication project

Walton, K; O'Callaghan, SM; Caris, S. Haemophilia Foundation Australia, Melbourne, Australia
W: www.haemophilia.org.au E: hfaust@haemophilia.org.au

AIMS

- To better understand the needs of young people with bleeding disorders
- To develop a web-based communication tool for young people
- To enable young people to connect, share experiences and obtain information about relevant life and life style choices, including work, travel, sport, recreation, relationships and socialising, while building the youth leadership capacity of Haemophilia Foundation Australia (HFA).

WHY THE NEED

- Treatment and care improvements have created a new generation with expectations of living a "normal" life
- Reports that many young people experience problems due to isolation, lack of knowledge about bleeding disorders or how to apply this information to themselves
- Difficulties engaging young people in planning for treatment and care services and in peer education and support.

WHAT WE DID

Needs assessment:
Questions - current issues, resources needed & resource preference
Consultation (face to face, email, phone)

- Young people affected by bleeding disorders aged between 13-30 recruited through facebook, newsletters, haemophilia treatment centres and local Haemophilia Foundations (this became the Youth Working Group)
  - Emailed most young people two questions per week to maintain engagement. Some preferred phone
  - Also asked what they do and don't like about websites
- Health professionals
- Parents recruited through newsletters and local Haemophilia Foundations
- Local Haemophilia Foundations.

Youth Workshop to explore web site design concept:
Two days, Melbourne-based; facilitated by HFA Youth Project Officer and other HFA educator; web site design company attended; 8 young people from Youth Working Group and 1 mentor participated.

WHAT WAS SAID

Needs Assessment Participants –
26 young people affected by bleeding disorders:
22 male, 4 female; 13-27 years; from all states and territories, except Northern Territory
16 health professionals (nurses, social workers/counsellors, physiotherapists)
9 parents
7 Foundations

SUMMARY OF RESPONSES

Concerns/Issues	Suitable employment options/preparation Independence when entering the adult health system/taking responsibility/self-management Disengagement from health care professionals Poor compliance with treatment Fitting treatment in with school/university Disclosure to friends/employers/schools Mental health (depression, anxiety, self-esteem, body image) Risk taking behaviour (physical activity; alcohol and drug use) Planning for travel Informed decision-making Learning what haemophilia is and what it means "Fitting in" with peers
Resource preferences	Social networking (facebook) Internet forum Handouts/leaflets Talking to peers Personal stories Group activities Mentor program Videos (YouTube)

YOUTH WORKING GROUP

"More visual – videos, pictures"
"Don't use medical mumbo-jumbo"
"What will happen during puberty and adolescence?"
"A good user interface which is easy to understand and navigate"
"Finding out about being a carrier"
"Video and video diaries would be great"
"Stigma of having a disease"


"The possibility of a cure"
"Tips for injections"
"Bold and in your face"
"Keep it simple"

"Use dot points"
"Real stories of others"
"Being isolated from friends"
"Not just haemophilia, not just boys"
"Emerging treatment options"
"Restriction of future employment"
"Educating my school and my classmates/friends with what the disorder is, and that I won't die if I get cut etc."

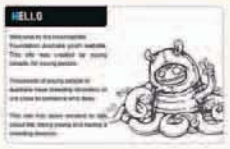
WHAT WE LEARNT

Young people want a web site to learn about living with bleeding disorders through each other's personal stories, that:

- Provides information in youth friendly language
- Provides opportunities to connect with each other through personal stories
- Provides mentoring through stories and comments
- Advertises current events – young people can get involved
- Encourages volunteering and fundraising.

Young people's stories and perspectives are *factored in* to education and support

Young people will have *factored in* the messages on the website before making life and lifestyle choices.



WHAT'S NEXT

New project to develop a leadership and mentoring program for young people affected by bleeding disorders:

- Takes into account the complexities of young people's communication, support and education needs
- Focuses on youth mentors and leaders developing content for the youth web site (Factoredin.org.au) and an ongoing national youth camp/workshop.

HAEMOPHILIA FOUNDATION AUSTRALIA

YOUTH NEWS

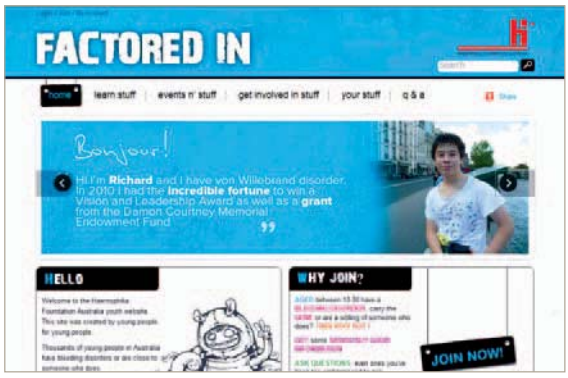
What have the Youth Working Group been up to? What do they think of the new Factored In website (factoredin.org.au)? What are they looking forward to?

"I like that it exists!"

"I think it is a fantastic way to interact"

"I have written a story, asked a question, made comments and voted on the poll."

"I like the look of the website. It has an appealing design. Very accessible. And the idea of the website that lets me decide if i want to ask (a question) at any time."



"I'm looking forward to getting more people involved. It will be good to see we can help people who might not be able to connect through other means."

"I like that it's Australian based and made for young people as well as adults"

"The website is a great way for young people with bleeding disorders to keep in contact, ask questions and find out about things that are going on in other states."

"I'm looking forward to the launch of the mentorship program."

"I have submitted a story about my personal experiences and have already had positive feedback from several other people telling me it has really helped"

"I'm hoping to be able to write some more stories."

"The website is easy to navigate and understand"

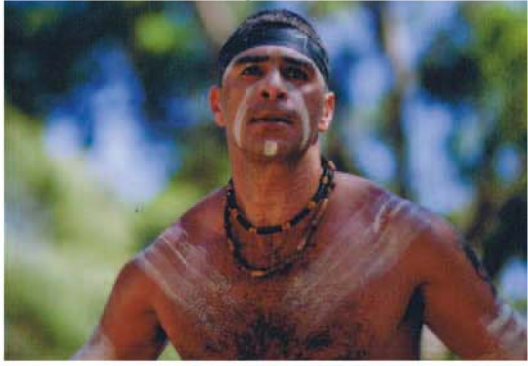
"I love the young and fresh feel of the website"

"I am looking forward to seeing some more stories, questions and participation from others."

"I have made comments and reviewed content"

FACTORED IN
WWW.FACTOREDIN.ORG.AU






SAVE THE DATE

MELBOURNE WELCOMES DELEGATES TO WFH 2014 WORLD CONGRESS

WFH 2014 WORLD CONGRESS
Melbourne, Australia • May 11-15






WFH 60 YEARS OF ADVANCING TREATMENT FOR ALL
WORLD FEDERATION OF HEMOPHILIA
Fédération mondiale de l'hémophilie
Federación Mundial de Hemofilia

MELBOURNE AUSTRALIA

HAEMOPHILIA FOUNDATION AUSTRALIA

CALENDAR

HAEMOPHILIA AWARENESS WEEK

7-13 October 2012
Tel: 03 9885 7800
Fax: 03 9885 1800
Email: hfaust@haemophilia.org.au
www.haemophilia.org.au

WORLD HAEMOPHILIA DAY

17 April 2013
www.wfh.org

XXXI INTERNATIONAL CONGRESS OF THE WORLD FEDERATION OF HEMOPHILIA

Melbourne, Australia 2014
www.wfh.org

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Haemophilia Foundation Australia (HFA) values the individuals, Trusts and Corporations, which donate funds to support our objectives.

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TEAM.FACTOR

2013 BUPA CHALLENGE TOUR ADELAIDE FRIDAY 25 JANUARY

Cycling enthusiasts from around Australia and the world are now registering to participate in the 2013 BUPA challenge tour on Friday 25 January. Cyclists will have the opportunity to ride the same route as competitors in the 2013 Santos Tour Down Under.

The route from Modbury, just north of Adelaide, to Tanunda in the beautiful Barossa will give cyclists the opportunity to challenge themselves just hours before the professionals take to the same route. The full route from Modbury to Tanunda is 127 kilometres, while the second start from Kersbrook is a 92 kilometre ride.

The third start from Mt Pleasant is 46.5 kilometres from the finish line at Tanunda.

For all you cyclist enthusiasts out there, join TEAM.FACTOR and participate in this amazing race. The team will be headed by Dr Simon McRae and will support Haemophilia Foundation Australia.

For registration and information visit www.tourdownunder.com.au/event-details.htm

When registering ensure you answer yes to being part of the team and the team code is BCT285

Will you be entering as part of a team? ○ YES ○ NO

What is the team code? BCT285

For more information contact
Dr Simon McRae
simon.mcrae@imvs.sa.gov.au
or phone 08 8222 6840

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