

# The 'H' Factor

Issue 34

Summer 2012

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## The President's Press

### BREAKING NEWS

**The Haemophilia Team at the Royal Children's Hospital are the recipients of a 2013 Australia Day Achievement Award for Children's Health Queensland. Congratulations team from everyone at HFQ!!!!**

Hi everyone,

Happy New Year to you all and I trust you all had a lovely break over the Christmas holidays and are all ready and rearing to go for 2013.

HFQ have a number of exciting events planned for 2013 including The Haemophilia Family camp in April and Haemophilia youth camp for boys in August, watch the mail for your invites and be sure to RSVP to Liz early so you don't miss out as numbers may be limited.

**OBE's lunches** will continue to meet the first Wednesday of every month and this year there will be the inclusion of a guest speaker at some of the events, if you would like to attend these lunches give Erl Roberts a call on 0421 915429 or Liz on 0419 706056 to RSVP. The first one for 2013 will kick off in February.

**Ladies luncheons** will resume on the 6th of March for mums, wives and those ladies who have a bleeding disorder. This luncheon is bi-monthly and includes a guest speaker who will address various topics appropriate to bleeding disorders. Give Liz a call if you would like to RSVP.

**Regional lunches** for 2013 will include lunches in Cairns, Townsville, Mackay, Rockhampton (TBC), Sunshine Coast, Toowoomba and Gold Coast. Dates for these will be advertised as they come closer and will be around the same time as clinic dates. These are great events for you to be able to meet others from the Haemophilia community within your area. Liz will have more information on the dates and venues of these events if you would like to email her on info@hfq.org.au to register your interest in attending.

**Haemophilia Foundation  
Queensland**

**HFQ Manager—Liz Fistonich**

**PO Box 451**

**Spring Hill**

**Phone: 0419 706056**

**Email: info@hfq.org.au**

**Facebook:**

**[https://www.facebook.com/  
HFQLD](https://www.facebook.com/HFQLD)**

**We are on The Web  
[www.hfq.org.au](http://www.hfq.org.au)**

# President's Press contd.

**Other events** that we are very excited about that are occurring throughout the year include our inaugural General Practitioner haemophilia training day, see page 10 of this newsletter for more information which we urge you to pass onto your GP if you think he or she may be interested in attending. We will also be running in conjunction with the Queensland Haemophilia Treatment Centre a range of seminars/meetings over the next 12 months including stress and sleep seminars, new family days, 25-35 year old educational luncheons and an aging seminar.

HFQ unfortunately has been the recipient of some Queensland health funding cuts so our fundraising calendar will be larger this year than ever before to ensure we are able to continue to be able to deliver the support we give to you all. In October we will be running another wheelathon, if families would like to attend or participate in this event email Liz who will be able to add you to our mailing list for information on this event. We will also be running several more Bunnings BBQ's this year and would love to have more volunteers around the Redcliffe area put

their hands up to help out.

For the next 12 months HFQ will continue to pursue activities that support the needs of the bleeding community, and will be providing representation at various forums so that our voice is heard as loudly as possible.

Regards

Howard Mitchell



## HFQ Christmas Party by Sarah Hartley (HFQ Secretary)

### Workshops Rail Museum, Ipswich

17 December 2012

The day started with a wild storm that hit Ipswich between 10 and 11am, which was unfortunate timing as this was when many of the attendees were on the road travelling to the venue. However, our HFQ community rises to a challenge, so people still managed to get there and join in the fun.

The Museum had a great children's playground area which was a big hit with the kids, and lots of other interesting exhibits. Lunch was a buffet with everything from wraps to quiches and spring rolls. The guest of honour Santa Claus made an appearance, initially without his hat as it must have blown off in the storm. Once his hat was found he gave the children some great early Christmas presents.

After lunch the sun came out which made it a bit easier and more pleasant for everyone to wander around the museum. Thanks to all who were able

to attend, your presence and good humour despite the weather made it a really enjoyable day!

Sarah Hartley



## ABOUT HFQ

The Haemophilia Foundation of Queensland (HFQ) provides advocacy, education and support for people in Queensland affected by inherited bleeding disorders. The Foundation employs a fulltime Co-ordinator and is guided by a voluntary Management Committee which meets monthly.

Members of HFQ are entitled to benefits, including subsidies on:

**Medic Alert bracelets (50% discount)**

**Electric Shavers (up to \$75 off)**

**Cool Relief patches (special trial offers)**

**Supportive footwear (75% off)**

## HFQ Management Committee

President	Mr Howard Mitchell
Vice President	Mr Erl Roberts
Secretary	Mrs Sarah Hartley
Treasurer	Mr Peter David
Members	Mr Craig Bardsley
	Mr Robert Weatherall
	Dr John Rowell
	Mr Adam Lish
	Mrs Eva Turek
HFQ Delegates to HFA	Mrs Leanne Stephenson
	Mr David Stephenson

## LADIES

**A good place to visit if you want to read some well-written personal stories of women with bleeding disorders or feel connected with other women worldwide.**

**<http://mygirlsblood.org>**

## Queensland Haemophilia Centre Contact Details

### ROYAL BRISBANE AND WOMEN ' S HOSPITAL

Dr John Rowell—Haematologist 3646-8067

Beryl Zeissink—Nurse 3646-5727

After Hours—Page Haematologist 3646-8111

Emma Patterson—Physiotherapist 3646-8135

Maureen Spilsbury—Snr social worker 3646-8769

### ROYAL CHILDRENS HOSPITAL

Dr Simon Brown-Haematologist 3636-9030

Joanna McCosker—Nurse 3636-9030

After Hours—Banksia Ward 3636-7472

Sarah Anticich 3646 7937

Mona Chong – Psychologist 3646-7937

## HAEMOPHILIA CLINICS

### RBWH

Appointments 3646-7752 or 3646-7751 or speak to Beryl

Haemophilia and Genetic Clinic—Dr John Rowell—  
Wednesdays 1.30pm

Haemophilia/Orthopaedic Clinic—Dr John Rowell and Dr  
Brett Halliday—9am every four weeks

### RCH

Phone Joanna about appointments

Banksia Outpatients—Dr Simon Brown—Thursdays at  
2.30pm

### OUTREACH CLINICS

**GOLD COAST HOSPITAL, TOOWOOMBA GENERAL  
HOSPITAL, NAMBOUR HOSPITAL, CAIRNS BASE  
AND TOWNSVILLE HOSPITALS:**

Book through Joanna at RCH and Beryl at RBWH

# HFA Report

Haemophilia Foundation Australia continues to advocate for those with bleeding disorders. They engage with all the players in the blood arena to ensure issues are raised and to ensure that we are not forgotten as a priority group. For example HFA made a number of submissions on the national HEP C & HIV strategies to highlight the needs of people with bleeding disorders particularly around their complex care needs of living with co morbidities. We have community members who carry a greater ill health load which is difficult for them to manage, especially when you consider pain, fatigue, mobility problems, bleeding and viral infections. This adds up to an overload with health conditions which creates a significant barrier to managing health issues like HEP C. Stop for a moment and think about your health – when did you last get checked for liver health with your GP or Haemophilia Treatment centre ... make an appointment today as tomorrow can bring you closer to major health issues.

Check out the youth site 'factored in' – a HFA initiative that is getting youth involved, communicating and having fun <http://www.factoredin.org.au/>

We will all be hearing more about the international Haemophilia world Congress in Melbourne May 11-15 2014. I encourage you all to attend, I came back from the event in May 2012 and can recommend attending as it gives anyone the ability to talk to

experts in their field from all over the world, get all the latest information on any bleeding related topic you can think of. It certainly cleared up a few questions that I had.

You know, it's easy to take our great treatment here in Australia for granted and I wonder what it would be like if HFA were not up at the front line making sure our bleeding issues are represented, from me to HFA ... a big thank you, great job and keep it up.

David Stephenson.



## RESEARCH STUDIES

Want to have your say about your or your child's treatment and quality of life? Research studies are one way to do that. Check out the new haemophilia research studies on the HFA web site and make your views and experiences known!

<http://tinyurl.com/HFA-participating-in-research>



# Physiotherapy Update

## Happy New Year to everyone,

On the note of the new year (and resolutions) it seems a good time to mention exercise and physiotherapy at the Queensland Haemophilia Centre. For all of us maintaining physical fitness is an essential part of keeping healthy and well. Some of the benefits include improved: heart health, bone strength, weight, mood, sleep quality, and energy levels. For those with bleeding disorders exercise can be of extra importance to manage issues like joint and muscle problems, but also have extra challenges. I would like to therefore encourage people with bleeding disorders wanting support in designing an exercise program or participating in some form of physical fitness to make contact. Physiotherapy support can range from management of a joint problem or other injury through to designing a training program for a specific sport or the gym. There are also options in appropriate cases for referral to hydrotherapy or an exercise class here at the Royal Brisbane and Women's Hospital. For patients of the Queensland Haemophilia Centre outside of the Brisbane area please remember you can still contact me for advice and that I can also liaise with and organise referrals to local services if necessary.



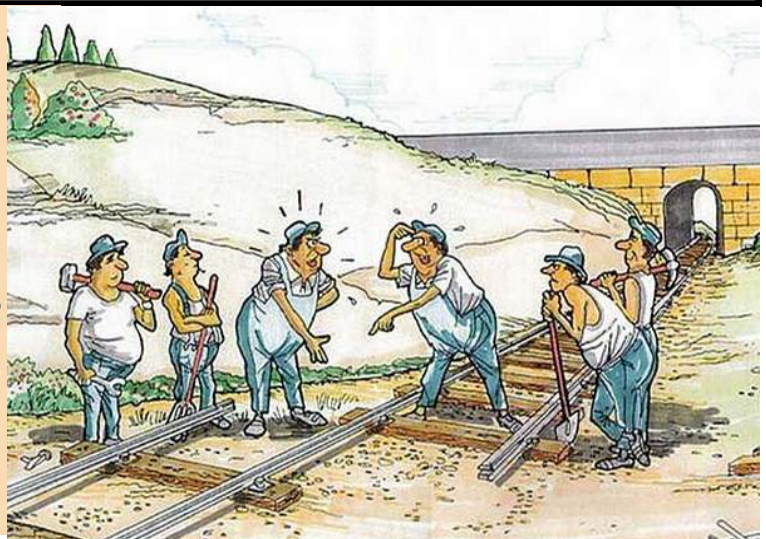
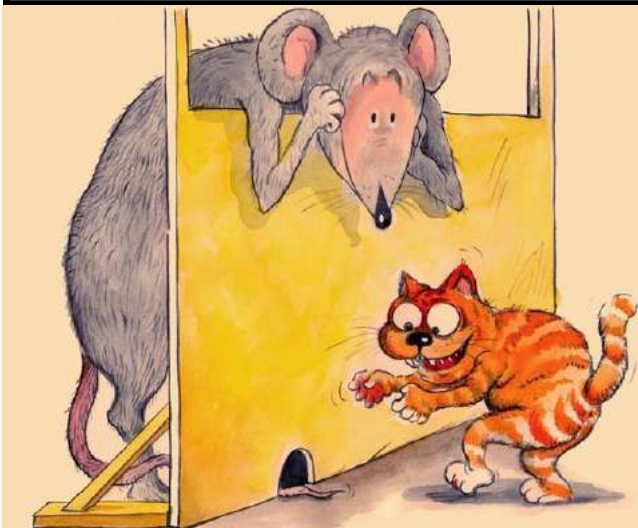
Those who are interested please contact me on (07) 3646 8135.

Regards,

Emma Paterson

Physiotherapist

Queensland Haemophilia Centre



# How to Get the Most out of Your Clinic Visit

By 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.

## **"Preparation is the key to success"**

*Alexander Graham Bell*

### **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?

## Contd.

### N e g o t i a t e   a   t r e a t m e n t   p l a n

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.

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.

# Social Work News RBWH



Happy New Year and welcome to 2013. I hope that you had an enjoyable Christmas with family and friends and that you are looking forward to the some of the exciting adventures that the New Year will bring. It would be foolhardy not to acknowledge that we will all undoubtedly, face new challenges as well. I am hoping that you have hope and confidence that you can deal with them as they surface. I like the question "How do you eat an elephant??" The answer ..... "Mouthful by mouthful!" It gives us hope and suggests that we can work at conquering barriers to contentment in our lives. I also like it because breaking down problems is a great way to make a problem immediately seem smaller and more manageable. Throughout the year we will be looking at ways of helping members of the community to "eat their elephants" and have a number of projects planned with that goal in mind. WATCH THIS SPACE for more information about informal sessions and group meetings over 2013.

There are a number of ways that we offer support and information. Feel free to give us a call, drop into our office or we can meet you somewhere closer to home for a chat. We can offer support related to haemophilia issues as well as referral for other needs. If

we don't have information about a situation we will look for it.

Areas in which we offer support include:-

- Support services at home - HACC (Home and Community Care) and ACAT (Aged Care Assessment) for assessment, information and referral regarding support services within your local area.
- Financial issues
- Centrelink
- Coping with pain and loss of mobility
- Work and study related issues
- Dealing with the impact of a bleeding condition on general functioning and relationships
- Blood borne virus issues
- Issues related to ageing
- Housing
- Patient Travel

For further information on any of these matters call

Maureen Spilsbury/Mona Chong on (07) 3646 8769

A quick reminder about our availability:-

**RBWH** – Maureen Spilsbury (Monday to Thursday – RBWH – ph 3646 8769)  
Advanced Social Worker

Mona Chong (Fridays only – RBWH – ph 3646 8769) - Advanced Psychologist

**RCH** - Sarah Anticich (Monday to Fridays – RCH – ph 3646 7937) – Senior Psychologist



# Social Work News RBWH

## Contd.

Are you interested in attending free **Cognitive Therapy Workshops** provided by the Hepatitis Council in 2013? Have a closer look at the flyer but remember that you need to book in by the end of January so don't miss the deadline if interested.

Have you heard about the **Men's Sheds Movement**? They have been running for years and have a great website which says that "Men's sheds are now established as part of the health infrastructure that supports programs to improve men's health and wellbeing".

It states that the **Vision for men's sheds is to:**

- Provide support to men in need of help arising from mental illness or other debilitating illnesses;
- Ease the transition of men from full-time employment to other activities in retirement;
- Provide an additional link between the primary health network and the many men who have no regular contact with that network;
- Establish a place for men that enabled social interaction and activities to maintain the health and wellbeing of those men;
- Build a culture in the sheds where all men are welcome and where mutual respect and trust are paramount;
- Become a focal point in the community for the identification of men's health issues and actions to resolve those issues.

They are Australia wide. There is probably one close to you. You can go to the [shed locator](#) to look at what is available in your local area.

Visit [www.mensheds.org.au](http://www.mensheds.org.au)

Please let us know if you need any information about these or any other matters.

I hope you have an exciting and victorious year!

Maureen Spilsbury

Advanced Social Worker – Phone (07 3646 8769)

### **Update - government changes re co-payments / prescriptions:**

Changes came into effect on 1 July 2012 that alter the amounts that can be dispensed, the corresponding co-payment and the number of months supply that can be on a single prescription.

Previously it was possible to receive a 3 month supply of a medicine for which you paid 3 co-payments. It was also possible to have an original prescription and 5 repeats, potentially allowing for 6 months supply on a single script.

Depending on how your doctor writes your prescription, it is now possible to receive a two month

supply of your medication for a *single* co-payment. Your doctor can also authorise up to 5 repeats, potentially allowing for 12 months supply on a single script - see your doctor about this.

# General Practitioner's Training Day for Haemophilia

HFQ in conjunction with the Qld Haemophilia Centre and the HIV & HCV Education Project at the University of Queensland, School of Medicine are planning a one day training forum for General Practitioners in March this year. Haemophilia Foundation Queensland is sponsoring the Forum which will be held in Brisbane. Please make contact with the Maureen Spilsbury or Mona Chong (07) 3646 8769 if you think your GP might be interested in attending. The forum will cover a range of health issues relevant to people in the haemophilia community. Below is the letter that has been sent to GP's—if you think your GP may be interested please pass this onto him.

**PATHOLOGY QUEENSLAND**  
*Royal Brisbane & Women's Hospital*  
*Division of Haematology*

**Enquiries:** Haematology  
**Telephone:** 07 3646 8067  
**Facsimile:** 07 3646 6319  
**Our Ref:** 13HA007  
**Date:** 7 January, 2013  
**Email:** [john\\_rowell@health.qld.gov.au](mailto:john_rowell@health.qld.gov.au)

*Dear Doctor*

*The Haemophilia Foundation of Queensland in conjunction with the Haemophilia Treatment Centre at RBWH and the HIV & HCV Education Project at the University of Queensland is conducting a one day seminar on Haemophilia, Viral Conditions and Ageing on Saturday 2nd March, 2013 at the Brisbane International, Virginia.*

*From our patient records – I understand you may be involved with a person with Haemophilia and invite you or someone from your practice to consider attending. Management of Haemophilia has progressed dramatically over the last twenty years with significant improvement in life span. Unfortunately complications from viral infections and aging may require specific management and broadening of care in the community and this seminar is directed at broadening knowledge in these areas.*

*There are a number of exciting and experienced speakers to present and it will be a wonderful opportunity for Haemophilia Centre staff to meet with those in community care and discuss these issues.*

*The forum will attract RACGP CPD points (2 points per hour).*

*There is no charge to attend the forum for Queensland residents and catering is provided.*

*Accommodation is available at the venue at a discounted room rate. Please liaise directly with the venue should this be a requirement for you.*

*With best wishes*

*Yours sincerely*

*Dr John Rowell*

# LADIES LUNCHEON AND INFORMATION DAYS

On a lovely summer day, the December's Ladies Luncheon was held at Vagelis Café and Bar in Hamilton. From my own personal experience having Glanzmann's Thrombasthenia (a platelet function defect) can sometimes be an isolating experience – as in 42 years, I've never actually met anyone else who suffers from the same blood disorder. But it was definitely a positive and uplifting experience to talk to women who either deal with family members with blood disorders, or suffer from other types of blood disorders.

Over lunch we compared symptoms and treatments and found that across the board symptoms and outcomes can be remarkably similar and treatments occasionally (although not always) crossover to assist more than type of bleeding ailment. **Who knew that these luncheons could be such a great source of information?** Likewise, we compared stories from hospitalisation's and doctor's appointments and laughed at some of the interesting situations we have occasionally experienced. Including one concerned roomie, who invited her evangelistic preacher and about 5 of his congregation to sing gospel songs to me in the middle of the hospital ward in an effort to cure me. Despite my skepticism, it did lift the spirits of the entire ward and provided some great story telling later. **Who knew that these luncheons are a great way of relieving stress and anxiety in meeting people experiencing similar things to yourself?**

In an effort to provide **YOU** with more information, support and networking so that we can all become better informed, we would looking to invite some exciting guest speakers to these luncheons. And although we have some great ideas, we would like some feedback as to whom **YOU** would like to meet, listen to and ask questions. So please contact us using the details below, **letting US know what we can do for YOU. Let your membership in HFQ work for you!**

We are also aware that for some of you, it is quite a distance for you to travel to Hamilton, HFQ is endeavouring to accommodate different venues to allow greater participation. Stay tuned to a new and improved calendar. Again, let US know how we can make it better for YOU.

EMAIL: [info@hfq.org.au](mailto:info@hfq.org.au), or phone 0419 706 056. All suggestions welcome.

Cheers

Eva Turek

NB: Ladies lunches are every 2nd month, with the first one for 2013 planned for March 6th. Please RSVP to Liz on 0419706056 or email [info@hfq.org.au](mailto:info@hfq.org.au) if you would like more information.



# Cartilage Grafting

**Researchers theories that if they can grow cartilage like cells in a lab it might be possible at some point in the future to graft these into the joints of people with Haemophilia.**

Traditionally cartilage grafting for people with osteoarthritis has been done by harvesting cartilage from a joint, however one group of researchers are looking to harvest cells from bone marrow rather than a patient's own cartilage. They are investigating the future possibility of grafting these into the joints of people with Haemophilia. Cartilage grafts traditionally need two surgeries. One surgery to harvest the cartilage which is then sent to a lab to be grown and then a second surgery to implant the graft. Some problems that might occur when trying to graft the joint of someone with Haemophilia include:

- 1) Whether the grafts would take.
- 2) If the underlying damage to the bone surrounding the joint was too bad it may not be able to keep the grafted cartilage alive and healthy.
- 3) Synovitis would not be fixed by cartilage grafting.
- 4) The area of damage to a joint with haemophilia is likely to be a spread out area, even if it is small as the damaging blood in the joint would have covered the entire surface of a joint. Usually a cartilage graft is only used on very small area's of damage, (ie to plug a small hole) not on joints where large area's are affected.
- 5) Bleeding risk.

The next stage of their research is to see if there are any problems with viruses being provoked or reactivated when they try to grow cartilage in the lab.

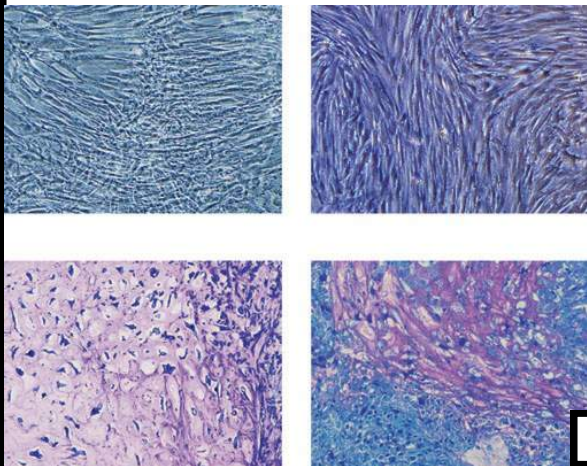


Fig. 1. Mesenchymal stem cells of BM from a haemophilia patient and a healthy adult. (a) Morphology of the cultured cells from a haemophilia and a healthy adult. They were spindle-shape like MSCs (9100). (b) Chondrocyte differentiation of BMMSCs from a haemophilia patient and a healthy adult. Cells were stained with toluidine blue and the stained cells were chondrocytes.

Fig. 1

*This article is a summary by David Stephenson of the original article written by Ebihara Y, Takadani H, Ishige I, Nagamura-inoue T, Wakitani S, Tojo A, Tsuji K. (2012) Feasibility of autologous bone marrow mesenchymal stem cells cultured with autologous serum for treatment of haemophilic arthropathy. (2012) Haemophilia: 1-3*



# Information from the Paris WFH

As many of you will be aware, David and Leanne Stephenson represented HFQ at the recent WFH congress in Paris. David has bought back many articles of interest for us to reprint into our regular newsletter, The 'H' Factor. Following is an interesting review of an article on Gene Therapy.

## Gene Therapy for Severe Haemophilia B

At the recent World Federation Of Haemophilia conference in Paris there was a presentation on Gene therapy by Edward Tuddenham ( University College – London) where he outlined progress on a trial involving gene therapy for severe Haemophilia B. The trial had three objectives – ***To understand the basis of gene transfer in haemophilia, To appreciate its inherent risks and benefits, and To grasp the future potential of this technology***

It was in the early 80's that gene transfer was first proposed. Back then it was thought it would be possible to fix the mutated gene and effectively provide a cure. That was a long time ago and clearly it was initially not so easy. As the technology developed we are now seeing some success and this trial involving 8 subjects now shows the safety and efficacy of this method or Gene therapy with subjects shown long term success factor IX expression.

Humans, unlike animals, have a more refined immune response which extends inside cells – so any delivery of a foreign particle inside a cell will normally be detected and destroyed. It is so much easier to cure haemophilia in animals as they don't have this intracellular immune response to introduction of a virus. Scientist eventually came up with a method to prevent this immune response inside cells from destroying the new DNA delivery which paved the way forward. A Viral vector was engineered after a great deal of experience with mice and non human primates and stage was set for the next step which was a human trial.

Safety is a mandatory requirement for any drug and the time frames are typically in years for this to occur. There were many questions and unknowns that needed answers, like where does the viral vector go after injecting, are there any side effects etc .... it turns out that over 90% of the infused product goes to the liver and there are no special procedures, it's simply injected into an arm vein and it finds its way to the liver.

The human trial used three dose levels (based on monkey data), there was initially no immunosuppression drugs used, patients had to be over 18 years old, must have severe Haemophilia B ( <1% factor IX), and importantly a lack of pre existing immunity to the AAV8 virus.

To be clear, this is not an integrating virus process where the DNA becomes part of your genome ( it does not go into the cell's own genome!) so there was no or little risk of cancer outcomes as a result of this method. Here is a summary of the 8 subjects. The trial is planned to recruit up to about 30 subjects as it moves forward.

When the first patient was infused, there was no adverse reaction, he stayed in hospital overnight and went back to work the next day. They checked to see what the virus was doing in the body and found that it disappeared from all body fluids after a few days, the liver function was closely monitored and the results for all 8 subjects follow:



## Gene Therapy for Severe Haemophilia B contd.

### **Subject 1: LOW DOSE**

Two years after infusion he has not gone below 2% factor IX level, he has stopped prophylaxis, has occasional trauma induced bleeds ( but these have o be significant traumas) and his factor usage went down by 77% with no liver issues.

### **Subject 2 & 3:LOW & INTERMEDIATE DOSE**

One of these two people has severely effected joints, he now has a new base line factor level of 2% and has reduced prophylaxis to once per week. The third patient also responded – scientists were hoping for 10% factor level based on the monkey data but this was not to be – this subject settled out at 2% - he now treats once every two weeks.

So the learning from the first three subjects is that you still need some treatment for significant trauma given a level of joint arthropathy.

### **Subject 4: INTERMEDIATE DOSE**

He has levelled off at about 2 to 3 %

### **Subject 5: HIGH DOSE**

This next subject at high level dose had some liver inflammation issues at week 7, however this was quickly fixed with a short course of steroids. His factor level settled at ~2%

### **Subject 6: HIGH DOSE**

Since infusion he has not had any treatment – that's ~ 21 month ago and he has stabilised at ~4% factor level and continues his sporting activities including marathon running and football on weekends.

### **Subject 7:HIGH DOSE**

This subject also has no further treatment and his base line has settled out at 4 – 6 %. He had the same reaction at ~ week 7 with liver inflammation but again this was sorted with a short course of steroids.

### **Subject 8: HIGH DOSE**

This subject has settled out at ~5% factor production.

An interesting fact is that you cannot use the AAV8 vector again – **it's a one shot deal** as the body detects it and has an immune response ready for any further exposure. So any future treatment with that vector would not work, however scientist have other vector that they know work that could be used sequentially eg AAV5 and others, so multi dosing is possible down the track.

So in summary 8 subjects have been treated – 2 with low dose, 2 intermediate and 4 high dose levels. There are no acute side effects, it's easy to inject and FIX expression is 1 – 6 % in all subjects between 3 and 24 months so far.

Out of the 8 subjects, 6 have been able to stop prophylaxis, 2 of 8 have reduced prophylaxis and evidence of improved quality of life in all 8 subjects – some have said '**life changing**' The next step with this trial is to have two higher dose subjects; they will then pause while making some new, purer vector which has a much higher percentage of effective product as opposed to the 90% empty delivery packets.

We now have proof of concept as a result of this trial and our thanks must go to the brave people who took a chance, and to the expertise and dedication of those involved in the making of this treatment.

Reviewed and summarised by David Stephenson.

## **Research Review** by David Stephenson

# **Stem Cell Transplant Appears to Clear HIV in Two Men on Treatment**

Two HIV-infected men are showing no traces of HIV in their blood after going through stem cell transplants similar to the one "Berlin Patient" Timothy Brown received, presented at the XIX International AIDS Conference.

The study results, discussed by Daniel Kuritzkes, M.D., from Brigham and Women's Hospital in Boston, showed a promising method of targeting the latent or hidden HIV reservoir.

Both patients underwent allergenic (or foreign) stem cell transplantation for the treatment of lymphoma after being on antiretroviral therapy (ART) for about four years. The first patient was born with HIV and started ART three to four years before the transplant, while the second acquired HIV through sex in the mid '80s and started ART in 2003 before receiving a transplant in 2007.

Prior to the transplants, both men had undetectable viral loads, but still had HIV in hidden CD4 reservoirs. In contrast to Brown, they received a milder form of chemotherapy just before their transplants and were able to stay on HIV treatment throughout the transplant process.

While Brown's donor cells lacked the key CCR5 receptor, which HIV uses to attach to CD4 cells, the two men received donor cells that did have the CCR5 receptor and were fully susceptible to HIV. However, because they were able to remain on ART during the transplant period, the donor cells were not infected with HIV.

"We believe that continual administration of effective ART protected the donor cells from becoming HIV infected as those donor cells eliminated and replaced the patients' own immune cells, effectively clearing the virus from the patients' blood lymphocytes," Kuritzkes said.

One of the men has been followed for two years and the other for three and a half years. While both are still on HIV treatment, neither shows traces of HIV in their blood plasma nor purified CD4 T cells using a sensitive culture method (less than 3 copies/ml). They are also showing a significant decline in HIV antibodies, suggesting a lack of HIV replication.

"The importance of our findings is that we have evidence now that we can protect uninfected cells from becoming infected when they're transplanted into an HIV-infected patient, a form of PrEP at the cellular level, if you will," Kuritzkes added.

The researchers plan on assessing the full extent of HIV reservoir reduction by looking for HIV in various body tissues and analysing the effects of treatment interruption.

<http://www.thebodypro.com/content/68300/is-the-berlin-patient-no-longer-alone-stem-cell-tr.html?ic=700100>

# Youth Project update

By **Kate Walton**

HFA Youth Project Officer

## FACTORED IN WEB SITE FOR YOUNG PEOPLE

### Competitions

Competitions will now be running on the Factored In web site regularly. Our first competition, the 'Achieving your dream video competition' was launched in October 2012 to run in conjunction with Haemophilia Awareness Week. Smaller competitions will also be run throughout the year quizzing young people's knowledge about the content on the website. 'Answer the question, Barry!' has finished for 2012 but keep a look out for more fun 2013. Featuring competitions on the web site was a suggestion from the Youth Working Group (YWG). They thought it was a way to have fun and encourage young people to stay involved with the Factored In web site community, and that this would help to build a strong youth presence in the bleeding disorder community.

### Information and personal stories

There are already some great resources available on Factored In including material on sport and medical information. There are many personal stories written by young people with bleedings disorders, young women who carry the gene or siblings who are willing to share their stories. Information on travel was posted up in December and we hope to have employment up next.

### Q & A

The Question and Answer section on the website has been quite popular. Users are invited to ask questions about bleeding disorders, and they are then passed on to the relevant 'expert' to answer. Depending on the question they may be answered by a haemophilia doctor, nurse, physiotherapist, social worker or a YWG member. As you don't have to be a member of Factored In to ask a question, anyone is able to use this function. When utilising this part of the web site, users are asked to provide their email address and a nickname. They are also asked if they want their question answered on the web site or if they want a personal answer via email. Both of these options have been utilised by users. Responses can take up to 2-4 weeks depending on the complexity of the question and the availability of the experts. Have a question about a bleeding disorder you've been busting to ask? Why not ask it on Factored In?

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### COMPETITION: QUESTION THREE, BARRY

For your chance to enter the draw to win 2 movie tickets answer the following question by Friday 14th December 2012 by 10 am AEDT.

Q3. What is first prize in the 'Achieving your dream video competition'?

\*Tip: you will find the answer on this website

\*Remember – once you have found the answer what it is to [technide@haemophilia.org.au](mailto:technide@haemophilia.org.au) with the subject 'Answer the question #3'. Make sure you include your nickname in the email

\* You must be a member of Factored In to enter this competition

Good luck!

Make a comment

RELATED LINKS

Competition Blog

- > Competition: Question one CLOSED
- > Competition: Question two CLOSED
- > Competition: Question three Barry

Q & A

What sports do you think I should play?

Everyone is different and has a different bleed history. Therefore you need advice that is individually tailored to you. For example, if you have no...

Read more

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### ACHIEVING YOUR DREAM VIDEO COMPETITION

NEWS FLASH! Due to popular demand the 'Achieving your dream video competition' has been extended until 31st December 2012 HAPPY NEW YEAR!

Haemophilia Foundation Australia is seeking entrants between the ages of 13-20 to enter our video competition 'Achieving your dream' to help raise awareness for Haemophilia Awareness Week. The Competition opens on Thursday 11th October 2012 and closes Monday 31st December 2012.

The video could be about a dream you have already achieved or one you still hope to achieve.

The prizes:

1st place: JVC GZ E105 Digital video camera and a 16GB memory card

TAGS

AH, appreciate events, beautiful, movie, video, communication, competition, care, film, fully, fundraising, give, growing, holiday, movies, movie, movement, parents, positive, question, 2, new, saving, self, giving, sibling, managers, television, travel, using, video, when, watching, work



# Youth Project update contd

## HFAS LEADERSHIP AND MENTORING PROGRAM FOR YOUNG PEOPLE

The findings of the needs assessment (Beyond Prophylaxis project: understanding and addressing the unmet needs of young people with bleeding disorders) led to the concept of "youth catch ups," (as compared to camps), as a model for the mentoring program. The aim of this model is for young people to have contact with each other more often, while not asking them to commit to too much time in the hope that friendships form naturally where young people exchange phone numbers, email, or become facebook friends etc.

Young people identified as leaders and mentors will play a large role in organising "catch ups" and will help make decisions such as where and how 'Catch ups" will take place, with the assistance of myself and their Foundation. Mentors and leaders will also have other roles such as moderating the Factored In website, writing comments and blogs for the website and promoting youth events in their state. The leaders and mentors will receive training from HFA on topics such as communication skills, privacy and confidentiality, event planning and promotion and web site moderation.

The leadership and mentoring program is an exciting project for HFA. Stay tuned for further updates.

# Mild haemophilia fact sheet



Although people with mild haemophilia may not have bleeding problems as often as others with moderate or severe haemophilia, this means that bleeding problems can be unfamiliar and people can be unsure about them.

How do you know what a "bleed" looks like? What do you do when you have an injury or bleeding? What does your GP or dentist or surgeon or obstetrics team need to know? What else do you need to know?

HFA has developed a short fact sheet on mild haemophilia to answer these questions. The fact sheet includes tips on managing mild haemophilia from people with mild haemophilia, women who carry the haemophilia gene and Haemophilia Centres.

The fact sheet has been developed and reviewed by two volunteer groups, one including people with mild haemophilia or women who carry the haemophilia gene, the other including expert haematologists and haemophilia nurses, social workers and counsellors and physiotherapists. Our thanks to the volunteer review groups who made a major contribution to the development of the fact sheet.

Print copies of the fact sheet can be ordered from HFA by emailing [hfaust@haemophilia.org.au](mailto:hfaust@haemophilia.org.au) or calling 1800 807 173. The fact sheet can also be downloaded from the HFA web site - [www.haemophilia.org.au](http://www.haemophilia.org.au) >Publications.

# Latest News from RCH

from Joanna

## Did You Know:

Your factor products are provided free of charge and are funded by the Federal and State Governments. The State Governments budget for your clotting factor therapy was devolved to the Children's Health Queensland and Metro North Hospital & Health Services during 2012.

Please remember factor products are expensive and every attempt should be made not to waste or throw out product unnecessarily.

Please do not double dose a bleed or increase your prophylaxis dose without consultation with your Haemophilia team at the hospital.

## Alert: Supplies to the Paediatric Community

From the 1<sup>st</sup> March 2013 supplies of consumables will no longer be provided by your local hospital free of charge!

This is a decision that has been made by the Hospital and Health Services across Queensland to meet budget constraints implemented by the State Government.

Staff are working hard to find other avenues of supplying consumables, but they will no longer be free. I am hopeful the hospital will be able to provide a range of solutions or schemes which will minimise the cost to families particularly those who use ports.

"Independence Australia" is one such online shop for health care items in the community. Please take a look at the link provided. [www.independenceaustralia.com](http://www.independenceaustralia.com)

We understand that this will cause a great deal of distress to our families and we are also concerned about the impact this will have on all of you.

I do not have all the answers yet, so I am asking if you could please not inundate me with phone calls. I will get the information to you when I receive it.

In the meantime if you are experiencing difficulties because of this change please choose a course of action to ensure your child still receives their prophylactic treatment, for example going to the Emergency Dept or Paediatric Ward to have your treatments. I'm sorry as we know that this is not a perfect solution by any means.

If you feel you will be adversely affected financially please contact us at [RCH-Haemophilia@health.qld.gov.au](mailto:RCH-Haemophilia@health.qld.gov.au) and let us know your situation.



