

the

FACTOR



Issue 46  
Autumn 2016

Newsletter of Haemophilia Foundation Queensland

## From the President



Hi everyone,

last edition I updated you on the incidence of inhibitors in previously untreated patients (PUPs) with Severe Haemophilia A. Many members are concerned

about inhibitors and their possible impact. This developing research is specific to previously untreated patients (PUPs) and to date there is no known increased risk for any other patients using these products.

Here is a very brief timeline on recent the investigations so far:

**Oct 3, 2014** You may recall the 'World Federation Of Haemophilia' (WFH) previously advised <sup>1</sup> a study published by a group in France demonstrating a higher than expected incidence of inhibitors in PUPs treated with Kogenate FS / Bayer / Helixazte NexGen compared to other

recombinant factor VIII (rFVIII) products. WFH requested that the US Food and Drug Administration (FDA) and the European Medicine Agency (EMA) examine all the relevant data and come to a conclusion as soon as possible. Both the FDA and the EMA confirmed that they will be re-examining the data.

**Oct 22, 2014** – A UK investigation <sup>2</sup> into the effect of recombinant factor (rFVIII) brand on inhibitor development was investigated in 407 severe haemophilia A PUPs born in the UK between Jan 2000 and Dec

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## Presidents Message *Continued...*

2011 indicated:

- Kogenate Bayer / Helixate Nexgen was associated with a higher inhibitor incidence than Advate in 407 consecutive UK severe haemophilia A PUPs
- Other risk factors for inhibitor development were factor VIII genotype, ethnicity and intensive treatment episodes

**Nov 11, 2015** – Some results of the SIPPET (Survey of Inhibitors in Plasma-Products Exposed Toddlers) study were published recently as a conference abstract<sup>3</sup> in advance of the American Society of Haematology (ASH) Annual Meeting. The abstract suggests that in PUPs, the risk of developing an inhibitor when using recombinant factors is significantly higher than when using plasma derived factor concentrates. This is an important study and the results may prove to be significant.

Remember this is in relation to previously untreated patients – PUPs based on the currently available published data. It remains the position of the WFH that it may be prudent to consider not using Kogenate FS / Bayer / Helixate NexGen for newly diagnosed PUPs with severe haemophilia A where other safe clotting factor concentrates are available. There is no known increased risk for any other patients using these products.

If you have any questions your Haemophilia Treatment Centre is the place to go

*David Stephenson*

President HFQ .....Advocacy, Education, Health promotion, Support

References:

<http://www.wfh.org/en/our-work/treatment-safety/inhibitors-pups-update-nov2014>

<http://www.bloodjournal.org/content/bloodjournal/early/2014/10/22/blood-2014-07-580498.full.pdf?sso-checked=true>

<https://ash.confex.com/ash/2015/webprogram/Paper82866.html>



## We asked Dr Rowell...

*We asked Dr's Rowell and Brown if they wanted to comment further on Dave's update about the occurrence of inhibitors in previously untreated patients. John replied with the following note of caution –*

In much the same way there might be differences in recombinant products, there can

also be differences in plasma derived products and one should be careful generalising. PD products are made in different factories with different processes and residual proteins in the products may vary. Also the SIPPETT study has been presented – but possibly one should wait for peer review / publication and comment from others – before making robust

conclusions. But – it is still very interesting and could have a significant impact on treatment

**Dr John Rowell**  
Director of Haematology,  
Pathology Queensland  
Director of Haemophilia  
Centre, Royal Brisbane and  
Women's Hospital

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## ABOUT HFQ

The Haemophilia Foundation of Queensland (HFQ) provides representation, health promotion, education and support for people in Queensland affected by inherited bleeding disorders. The Foundation employs a part time manager and is guided by a Board of Directors which meets monthly.

We can be contacted on mobile 0419 706 056; or via email ([info@hfq.org.au](mailto:info@hfq.org.au)) or post at PO Box 122 Fortitude valley, Qld 4006

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

- ◆ **Medic Alert bracelets (50% discount)**
- ◆ **Electric Shavers (up to \$75 off)**
- ◆ **Supportive footwear (75% off)**
- ◆ **Discounted Movie Tickets**

## HFQ Management Committee

President	...	...	Mr David Stephenson
Vice President	...	...	Mr Adam Lish
Secretary	...	...	Mrs Leanne Stephenson
Treasurer	...	...	Mr Peter David
Members	...	...	Mr Robert Weatherall
			Mrs Sarah Hartley
			Dr John Rowell
			Mr Erl Roberts

### HFQ Delegates to HFA

Mr Adam Lish & Mr David Stephenson

## Acknowledgements

HFQ is grateful for the support of our patron: His Excellency the Honourable Paul de Jersey AC .

HFQ programs and services are funded by the Queensland Government.

## Internet

Find us on the web at [www.hfq.org.au](http://www.hfq.org.au) or at our Facebook page at [www.facebook.com/HFQLD](http://www.facebook.com/HFQLD)

## QUEENSLAND HAEMOPHILIA CENTRES

### CHILDREN'S CLINIC

#### PAEDIATRIC CLINIC STAFF (LCCH)

Switch: 07-3068 1111 Haemophilia Mobile 0438 792 063

Dr Simon Brown — Haematologist

Haemophilia Registrar Dr Melanie Jackson

Joanna McCosker – Clinical Nurse Consultant

Wendy Poulsen — Physiotherapist

Moana Harlen — Senior Psychologist

**Contacting the Clinic** Please call the mobile for urgent enquiries (during office hours only). For all non-clinical/non-urgent enquires please email [LCCH-Haemophilia@health.qld.gov.au](mailto:LCCH-Haemophilia@health.qld.gov.au)

After hours—call switch and ask to speak with on-call haematology consultant or present to the emergency department

Appointments — Contact the Administration Officer for Haematology or 2e outpatients for queries regarding clinic appointments

Haemophilia Outpatient Clinic — Dr Simon Brown — held in 2e outpatients Level 2, Thursday afternoons 1.30 – 3.30pm

Contact the Administration Officer for Haematology Department

### ADULTS CLINIC

#### ADULT CLINIC STAFF (RBWH)

Dr John Rowell — Haematologist 3646-8067

Beryl Zeissink — Clinical Nurse Consultant 3646-5727

Alex Connolly – Clinical Nurse (Part time) 3646-5727

After Hours — Page Haematologist 3646-8111

Rebecca Dalzell — Physiotherapist 3646-8135

Michael Hockey — Physiotherapist 3646-8135

Loretta Riley — Advanced Social Worker 3646-8769

Desdemona (Mona) Chong – Advanced Psychologist (Alt Tuesdays and every Friday) 3646-7937

**Contacting the Clinic** Please telephone in the first instance.

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

## OUTREACH CLINICS

Gold Coast Hospital, Toowoomba General Hospital, Nambour Hospital, Cairns Base Hospital & Townsville Hospitals: Book through Joanna at LCCH and Beryl at RBWH.

## Building Resilience - workshop report

Building Resilience through Mindful Practice was the second workshop in the RBWH Life Series Workshops.

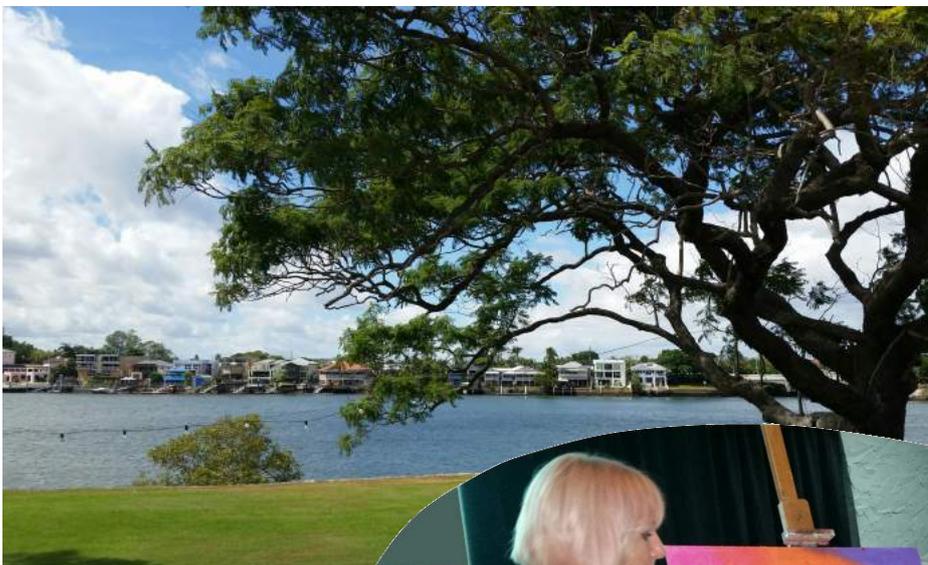
It was held on 4 Mar 2015 at picturesque Riverside Receptions. The focus was on building resilience through mindful awareness. Unlike the first workshop where a mighty storm visited us, weather was perfect this time (We got that one sorted this time!).

scientific evidence for the benefits of doing mindfulness. This is important as we all need to understand why we are encouraged to do certain things and know that there is evidence backing up what we do.

The segment which resonated most with participants were the two experiential mindfulness exercises. In the first exercise on mindful breathing, participants were taught how to focus on their

differently as they attempted to draw basic shapes and lines. We learnt to see how things relate to each other, create balance and even train our perception to focus on form and structure.

Overall, there were many learning gems, and it seems everyone took home something different. Some wanted to know more about mindfulness and others were inspired to take art classes!



With the Brisbane river just outside the training room (see photo), it was not difficult for participants to slow down and focus on the contents of the workshop.

Our presenters were Dr Ira van der Steenstraten (life coach) and Priscilla Jean (visual artist). Together, they put together materials to impart their knowledge about the role of mindfulness in building resilience and the unique way that art can help to develop our creative awareness. Contents include first understanding what resilience is and what factors may contribute to one's resilience. These included things like social support, having a role model, humour and training. Training is where learning mindfulness practice sits. Then, participants learnt about the theory and

breath and allow their thoughts to come and go as they pay attention to their breath. In the second exercise, participants were guided to use their five senses (one at a time, sight was the last to be used) to eat a grape mindfully.

Creative awareness using art follows after lunch. Drawing was a relatively new concept for many participants and participants were challenged to look at things

If you are interested to find out more about the contents of the workshop, please contact Mona ([desdemona.chong@health.qld.gov.au](mailto:desdemona.chong@health.qld.gov.au) or 07-3646 7937) who will point you in the right direction depending on your query. Also, planning for the next workshop is underway and the focus would most likely be on managing chronic pain on a daily basis. Please indicate your interest as early as possible so that Mona can develop a workshop that best caters to your needs.



**Please Note:** The Life Series workshops were made possible through support from the Haemophilia Foundation Queensland and funding from a Changing Possibilities in Haemophilia Grant by NovoNordisk,

## New Social Worker at RBWH

### Greetings from Loretta.

Graham asked if I would like the opportunity to introduce myself to the HFQ. I'm the newly appointed Social Worker in the Queensland Haemophilia Centre; currently also known as the 'new Maureen'. I studied Social Work at the University of Queensland and have been a Social Worker for over 20 years, working in a variety of settings in Queensland (rural, regional and metropolitan), Victoria, the United Kingdom and Ireland. I have experience working in areas including Mental Health, Hospital and Community Health settings, Palliative care and Child Protection.

My experience in Social Work has given me an understanding and knowledge of the impacts of chronic illness and strategies which assist people cope with life stressors. I also have networks

into many other services that may be able to help. Additionally, I have a strong interest in grief and loss, mental health (including preventative mental health) and rural/regional issues, having grown up and later worked in regional and rural/remote areas of Queensland.

March 15th was World Social Work Day. '*Forging Solutions out of Challenges*' is the theme adopted in 2016 by the United States of America National Association of Social Workers for the month of March. The reason I mention this is that it summarises part of the framework in which I practice.

My hope for my position in the Queensland Haemophilia Centre is to work together with you towards developing solutions to assist with challenges you may face. Together we may be able

to come up with a solution, an avenue to progress a solution or a different way of viewing the challenge which may bring about its own solution.

I would like to share a heartfelt appreciation to everyone who has made me feel welcome in my first few weeks, sometimes sharing their stories, which has given me the privilege of developing an understanding of each person's own personal journey.

I look forward to meeting many members of the community in the future, through the clinics, at groups and workshops or when you visit the Queensland Haemophilia Centre.

*Loretta*

## BOOSTER CLASSES

IN THE RBWH PHYSIOTHERAPY DEPARTMENT

Come on the **first Tuesday of every Month** from 1 - 2pm for a variety of exercise and wellness classes to boost both your knowledge and physical wellbeing.

Each month you should come away with new skills (or be reminded of old ones) to continue at home, and the exercises prescribed will be specifically designed to have the best physical outcomes for men with bleeding disorders.

**PHYSICAL ACTIVITY IS ESSENTIAL FOR BONE MUSCLE AND JOINT HEALTH.**

Classes will include **HYDROTHERAPY**, **STRONG BLOODY MEN** (the first series was a hit), **GRAVITY FIT** (if you don't know what this is then you definitely need to be there!) and **BACK EXERCISE CLASSES**.

Please RSVP for more details to;

Bec Dalzell, Physiotherapist, Qld Haemophilia Centre  
P: (07) 36468135 E: rebecca.dalzell@health.qld.gov.au

**MAKE THE EFFORT AND SEE THE BENEFITS!**



## From the Paediatric Team

Here is the latest news from the Qld paediatric Haemophilia Centre...

### **Calling for expressions of interest to participate in Skype research.**

We are about to begin a new research project that aims to investigate the usefulness of using telehealth (skype) for providing multi-disciplinary assessment and advice for children with haemophilia. The aim of this is to hopefully show that review by Skype is an appropriate, effective and feasible tool to assess a suspected bleed. By adding a visual component to assessment we hope to reduce the need for you and your child to present to hospital for review by the team. For participation you will need to have the ability to connect to skype so you will need a laptop, pc, tablet, or a smart phone and internet connection.

The plan is to obtain parental consent to participate prior to an acute bleeding episode and subsequent phone call for advice. Study information will be provided with written details and information for connecting with the HTC using a skype connection.

If you think you may be interested in participating please contact Joanna or Moana at [LCCH-Haemophilia@health.qld.gov.au](mailto:LCCH-Haemophilia@health.qld.gov.au).

### **Welcome to our new staff at the paediatric HTC team.**

Welcome to our new fellow Dr Malaika Perchard who will be with us until August 2016. When Joanna is unavailable or nursing staff cannot take your clinical call then you may need to contact Dr Malaika via 3068 4403.

Welcome to Hayley Coulson who will be working with Wendy in physiotherapy. Hayley has

already provided cover whilst Wendy was on holidays and will also provide outreach clinic support. She brings a breath of enthusiasm to our team"

Finally Amy Finlayson will be covering for Joanna McCosker every Monday until Joanna has completed her Nurse Practitioner course sometime in 2017.

### **Medic Alert application forms**

If you require a medic alert form they are online <https://www.medicalert.org.au/>

Feel free to download, print, complete and mail or scan and email to LCCH for completion by a doctor. (Ed—HFQ will cover 50% of members costs.)

### **LCCH email**

For a timely response to all non-urgent clinical issues and all supply or school requests please send an email to [LCCH-Haemophilia@health.qld.gov.au](mailto:LCCH-Haemophilia@health.qld.gov.au).

## Bleeding disorder Awareness

### **This month is World Haemophilia Day and we've been working hard to get the word out about ALL Bleeding Disorders.**

Many of our members will be familiar with April 17, the day the World Federation of Hemophilia (WFH) has declared as World Haemophilia Day (WHD). This year it is focused on bringing attention to a challenge that too many people in the bleeding disorder community face: lack of access to treatment and care.

It is a perfect opportunity to raise awareness about hemophilia and other inherited bleeding disorders, but also to consider how you can contribute to improving the lives of those living with one.

There is an enormous difference in the level of care available to patients with a bleeding disorder in other parts of the world. While

we can access medical care throughout our lives, most can not. Treatment works, but without the correct care many patients still suffer debilitating pain, joint damage, or death.

By marking World Hemophilia Day we can all play a part in reaching the shared goal of seeing every person with a bleeding disorder diagnosed and treated.

HFQ member Brett Williams has organised for the state MP's to receive and wear a badge promoting support for bleeding disorders on the closest sitting day to WHD.

You can also do your part to raise awareness and increase support for those living with an inherited bleeding disorder. Download the poster ([www.wfh.org](http://www.wfh.org)) and other WHD information and ask your library, school or workplace to display it.

World Hemophilia Day provides an opportunity for you to talk to your family and friends, colleagues, and caregivers.



Consider telling the local paper or radio station your story and why it is still a crises for people diagnosed with a bleeding disorder and what having support can do for you and in helping all people with bleeding disorders live successful lives.

## Ultrasound helps treatment & diagnoses

Magnetic resonance imaging (MRI), is considered to be the gold standard for musculoskeletal imaging, but musculoskeletal ultrasound (MSKUS) may be a better alternative for people with bleeding disorders who have joint disease, particularly in those with early joint changes.

Studies have predominantly looked at children with haemophilia. MSKUS has been shown to detect early changes in joints, including those that may be clinically asymptomatic. MSKUS may even be able to differentiate between arthritic pain and a bleed, however this requires a certain level of skill on the part of the clinician, and may be more difficult with advanced arthropathy.

Recent studies have found patients' with haemophilia were only correct with their perceptions on the cause of their pain one-third of the time. This is because there are many reasons why someone could have a swollen, painful, hot joint. It has been suggested that this is where musculoskeletal ultrasound (MSKUS) may have a significant application. The differentiation of the source of patients' pain may have a significant impact on the way we assess and manage bleeds and pain in haemophilia.

MSKUS also has a role to play in imaging muscle bleeds, determining their depth, size and location & tracking their progress. Currently the RBWH has access to MSKUS through the radiology department but the physio department are hoping to get one soon and Bec and other physios will then be trained in its use.

### Understanding ultrasound

MSKUS has been a standard practice in orthopaedic medicine and sports medicine for years but only recently has it been used for people with bleeding disorders. To take images of a joint, a gel is applied to the skin to help a probe gently glide over the area. The probe bounces sound waves off structures in the joint which are

then reflected back. The MSKUS unit converts these echoes into electrical signals that appear as images on a screen.

Ultrasound machines can make ligaments and tendons visible. MSKUS will also be able to document changes in cartilage resulting from osteoarthritis and/or synovitis, chronic inflammation of the synovial membrane surrounding the joint. MSKUS units may also be able to detect inflammation and abnormal blood flow.

You can distinguish how much a tendon and ligament has ruptured or how much the fibres have been disturbed, from partial- to full-thickness tears with MSKUS which can also pick up undiagnosed tears and sprain in about 10% of the scanned joints.

MSKUS has advantages over MRI, including the speed with which results are ready. Its sensitivity is also a plus. MSKUS is sensitive to soft tissue changes and bleeding, and it's not like an MRI, which is noisy and annoying, this is smaller and usually more portable with the patient often lying down for the procedure. A full joint exam using MSKUS takes 10–20 minutes whereas an MRI can take three to six times longer.

### Limitations

MSKUS does have limitations. It won't be able to go to the interior of the joint and can't detect things like anterior cruciate ligament tears. It can't penetrate bone, and it's not foolproof in pinpointing every source of pain. It is very clinician-dependent, and may have a limited role in joints that already have advanced degenerative changes. Lastly, these units are costly. The QHC will not have their own machine for some time and will have to borrow the physio one when it arrives.

### The future

Once the physiotherapists have the equipment and training, MSKUS will have a role to play in enhancing health outcomes. Scans may be done at your clinic visit, with results available immediately. Images may assist in directing treatment regimes, tracking progress, and setting appropriate exercise regimes.

The use of the ultrasound



technology is a potential win-win for both patients and their doctors. It can help both patients and clinicians distinguish the difference between the pain of having a bleed and the pain of having some arthritis or tendinitis- changes. For patients who are used to living with arthritis, MSKUS can also help you determine treatment changes. You should be able to see if there is any fluid along these borders or if the joint has telltale signs of being a bleed and your clotting factor dosage can be adjusted appropriately.

MSKUS may also be able to be used proactively and preventively in children during their annual QHC visit. If they have any signs of arthropathy (joint disease), then the clinic team can become more aggressive with their prophylaxis to prevent further arthropathy.

Edited for size and Queensland situation by the H Factor editor in liaison with the RBWH physiotherapists. Based on an article in Hemaware by Sarah M. Aldridge <http://www.hemaware.org/story/scans-demand?tr=y&aid=16435852>

## How problematic is Cannabis?

Cannabis is the most commonly used illicit drug in Australia. It's illegal right now, but there is talk about it being offered medicinally in the future. But what does it do to your health?

The potential harms associated with using cannabis depend on two things above all others.

- i) The age when you first use cannabis (especially if you're under 18). Cannabis use can impact on the key stages of brain development such as neural connections and the development of white matter.
- ii) Your pattern of use: the frequency, dose and duration makes a big difference, especially if you're using it weekly.

Research has shown that driving while cannabis-impaired doubles your car crash risk and that 10% of regular cannabis users become dependent. Regular cannabis use in adolescence also doubles the risks of early school-leaving and of

cognitive impairment and psychoses in adulthood. Cannabis smoking probably increases cardiovascular disease risk in middle-aged adults but we are not yet certain.

Even if you control for other variables these associations persist but this conclusion is challenged by other research. What we can conclude is that cannabis use is associated with these problems and may have contributed to the outcomes.

### Mental Health

Many studies on the relationship between cannabis use and mental illnesses are also not conclusive. The relationship between cannabis use and depression and anxiety disorders is complex. In contrast, the relationship between cannabis use and risk of developing symptoms of psychosis has been well established in many different review articles.

Early and frequent cannabis use can cause psychosis. We also know that cannabis use by people with established psychotic disorder can exacerbate symptoms.

The risk of developing schizophrenia increases with the duration and dose of cannabis use. If you've used cannabis at some point there is a 40% increased risk which doubles for regular users compared with non-users.

It is important to view all cannabis risks in context. As a general rule avoid all drugs and alcohol until the brain has stopped growing and if you can't keep off the grass, perhaps the best advice is the grandmother's mantra "everything in moderation".

If you have problems or concerns about cannabis, visit [www.ncpic.org.au](http://www.ncpic.org.au) or call the free national Cannabis Information Helpline on 1800 30 40 50.

## Experiencing the Mindfulness Workshop

With a little juggling work and organizing kids & school pick-up, me and my husband and 2 of our adult daughters and a good number of our great Haemophilia community attended the RBWH 'Resilience Workshop' run by a fellow Haemophiliac, Dr. Ira van der Steenstraten and co-hosted by Priscilla Jean, a visual artist.

I thoroughly enjoyed All of the workshop. The Finger Grips\* were fun and the Art was fascinating. It was a well-balanced, interesting program: the social, the food, the view, the learning, the presenters, the day, the time and the relaxed atmosphere were all interesting and positive for me. It was a beautiful way to end off the week.

Because of the Workshop, I decided to practice 'Mindful Breathing' to help me stay focused and sometimes I also do 'Mindful Eating' - eating slower, taking time to enjoy the flavours and textures, chewing more and making more saliva, (a healthier approach to eating).

In the second session - An introduction to Art was an amazing concept, relating 'Art' to Mindfulness. Fascinating learning how looking at life in a more complex and meaningful way. Everything has a bigger purpose than we think. How we interpret something will show in the quality of the outcome.

I have also tried to listen to the tone of my voice to make the right impact with the - 'Yes' 'No' concept\*\*. I am becoming more aware of listening to 'How' I says things. Instructions didn't always soak in to get the total understanding of some topics, but with reviewing the handouts info and practice, I will get better and better at being more aware, alert and focused.

In the future we are hoping for a Workshop using Mindfulness to help us to become more Resilient to Pain and Trauma. This would be very relevant to all of us and going by this recent workshop – Fun, Socially relaxing and empowering experience. Please, I strongly recommend attending the next workshop. (watch this space!)

Kind regards  
Lynn and Robbie Weatherall :)

\* For more information on Finger Grips (Chinese Finger Traps) see: <http://www.portlandpsychotherapyclinic.com/counseling/blog/chinese-finger-traps-what-novelty-item-can-teach-us-about-acceptance>

\*\* Noticing how saying "yes" and "no" impacts differentially on our emotional and physical states, then changing the way we speak to ourselves.

## A Safe Play Space in Hervey Bay

### *If Life wants to be challenging .... It often will!!*

Our son Bryden has severe haemophilia A and as life would have it after a simple fall at my baby shower we were sent on a whirlwind of a journey last year. After hitting his head we treated Bryden with an extra factor dose via his port on our kitchen bench. He was doing fine until two days later Bryden becoming floppy in the trolley at the local supermarket. We were unsure if he was experiencing a bleed from the earlier head injury or if something else was wrong?

Was it another port infection as he had one earlier that year? We took him to the local hospital for review and a CT scan but a few hours later he had a febrile convulsion



and went into septic shock . He was then airlifted to Brisbane's Lady Cilento Children's hospital for specialist care. It was discovered that Bryden did indeed have another infection in his port and it had now spread to his general blood circulation making him incredibly unwell. He was immediately rushed for surgery to have his port removed and antibiotics started to kill the infection pulsing through his blood stream. This would be the first of three surgeries for him.

### **The community rallies**

Meanwhile back in our hometown of Hervey Bay the community rallied around with a goal of creating a safe play space for Bryden and other children with Haemophilia. Local Business Coach Steve Baker launched a campaign to carry out a backyard

blitz for us. Our local member Ted Sorrenson and the RSL branch donated funds, whilst local business owners from Business Networking International (BNI) donated their time and resources to build a play space.

Our goal for Bryden has always been to allow him to live life to the max and in time learn what his body can and can't do.

However when you are only 2 years old this learning is often challenging.



Dr Simon Brown we were very proud to be able to hand over a cheque for \$1200 thanks to the left over funds from the local business group BNI.

### **Open home**

Now it's our desire is to share this play space with other Haemophilia families. Here they can visit and the children can connect in a safe environment. If you are interested in connecting with us and visiting us here in Hervey Bay we would love to hear from you.

You can see more of our journey at Little Aussie Bleeder on;

*Facebook* <https://www.facebook.com/littleaussiebleeder>

### **More trouble**

The morning of the backyard blitz we were sent into another tailspin when I suffered a major haemorrhage due to 'placenta percreta' and subsequently was rushed for surgery to deliver our little girl ten weeks premature. With 3 family members in hospital at the same time the team working on this surprise backyard renovation launched into action fuelled by the desire to bring us some happiness on our return home. Little did we know what they were up to?

### **The big reveal**

Two months later the day came for Bryden and I to be surprised by the backyard renovation. As any kid would be, Bryden was ecstatic at what he found. Here we will create memories that are full of joy, laughter and less haemo stress. This place is a haven for a haemophilia's mums sanity...helicopter mum isn't required and Bryden can be the kid he needs to be and for that I am grateful beyond words.

After recently officially opening the play space with local news crews attending and even our very own



*If you're excited by Bryden's play space and would like to visit Dion and Laura so your own children can have a play, please talk to Maona who can help connect you and may be able to help with getting there*  
- Ed

## Can you solve this riddle?

The answer is something to do with bleeding disorders.

I rush to the scene  
To save the day  
The help I provide  
makes it safe to play!

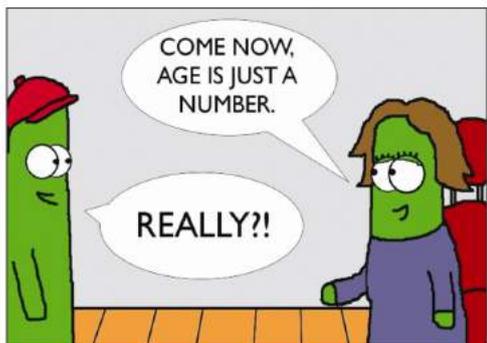
What am I? \_\_\_\_\_

## The Slobs

by Conor Birkett

First published by  
the Irish Haemophilia Society.

[https://www.haemophilia.ie/content.php?id=7&article\\_id=698&level3\\_id=779](https://www.haemophilia.ie/content.php?id=7&article_id=698&level3_id=779)



## Home Alone

Now that your parents or grandparents are trusting you to stay home alone, you're growing up! But if you feel a little worried, that's normal.

First: Ask your parents what they expect and what activities you're allowed to do. When you're at home, follow the house rules, and call your parents or emergency contact with questions or if there's a problem. Your emergency contact may be that nice neighbour who lives two doors down, a relative or a friend's parent. If you prepare yourself, and follow these tips, your time at home alone will be a breeze.

### Keep your key in a safe place

Put your house key on a necklace or key chain. If it's on a key chain, put it in a pocket that you can close, or in your backpack, so you don't lose it. Don't show the key to anyone or tell anyone you'll be home alone. If your garage door has a code to open it, memorize it and keep it a secret.

### Check out your surroundings

If anything feels strange or looks weird when you get home, like if the door is open or a strange vehicle is parked outside, don't go inside. Go right to your emergency contact's home instead. But if everything looks fine, good news. Just lock the door behind you or close the garage door when you get inside.

### Call to check in

Even if you *just* talked to your parents or grandparents after

school finished, they still want to make sure you get home safely every day. So give them a quick call to say you're home. If you're feeling any joint pain, or have a nosebleed or another health problem, speak up. They can help - even over the phone.



### Have a snack (maybe)

Talk to your parents about what you're allowed to eat and drink, and how you should prepare it. Don't use the oven, stove, or microwave because they can be dangerous. Your parents may want you to have snacks that don't need cooking or heating. Think crackers and cheese, fruit, or veggies and dip. Maybe even a sweet treat if you have permission. (But just one or two!)

### Start your routine

You and your parents should agree on what you're supposed to do every day, including having that snack and starting your homework. If chores are on that list, check them off after they're done. The point is to follow the routine from your parents. And remember: You're home alone, but not completely alone. Call your parents—or your emergency contact—if you have questions about *anything*. Then enjoy your time to yourself.

By Leslie Quander Wooldridge. First published in Hemaware Nov 2014 <http://www.hemaware.org/story/home-sweet-home-alone>

# Activity Page (The answers are on page 13)

Use the words in the box to fill in the blanks and complete the story.

### Word Box

Compression	elevation	factor	haemophilia	hobbled	ice
infusion	knee	rest	safe	Swings	swell

Kobe and his older brother, Brandon, were at the park playing on the swings.

*"Want to see what I can do?"* asked Kobe . He started to swing harder and harder, going higher and higher. Suddenly, he yelled **YAHOO!** And jumped off the swing, flew through the air, and landed on the grass!

Brandon shouted, *"Kobe, you really shouldn't do that!"*

*"Why not? Are you saying I shouldn't jump because I have \_\_\_\_\_?"* Kobe asked.

*"No, of course not."* answered Brandon. *"You shouldn't jump like that because it isn't \_\_\_\_\_ for anyone."*

Kobe ignored his brother and jumped off the swing again. This time though, he hit the grass, landing on his left \_\_\_\_\_.

*"Yeikes! That really hurt!"* cried Kobe .

Brandon quickly ran to help him, *"Your knee is starting to \_\_\_\_\_! We'd better hurry and get you home so mum can give you an \_\_\_\_\_ of \_\_\_\_\_."*

As the brothers \_\_\_\_\_ home, Brandon said to Kobe , *"When we get home you'll need to use R.I.C.E. Do you remember what that stands for?"*

*"I know, I know,"* mumbled Kobe, *"\_\_\_\_\_, I \_\_\_\_\_, and \_\_\_\_\_."*

Hey, and one more thing, 'Brandon added with a smile, *"no more jumping off the \_\_\_\_\_!"*

### Hidden Words

In each row of the first column there is a numbers box with a hidden 'bleeding disorder' word jumbled inside. In the box number 1 the word 'needle' is hidden but it matches the open squares of the box "E" in the second column. See if you can match each box in the first column with the boxes in the second column so that the hidden words are revealed?

1 

D	N	E	B	N	E	D	X	L	E
---	---	---	---	---	---	---	---	---	---

A 

--	--	--	--	--	--	--	--	--	--

2 

F	W	A	D	V	C	T	O	R	E
---	---	---	---	---	---	---	---	---	---

B 

--	--	--	--	--	--	--	--	--	--

3 

M	V	R	E	L	I	P	N	A	Y
---	---	---	---	---	---	---	---	---	---

C 

--	--	--	--	--	--	--	--	--	--

4 

K	I	N	G	F	U	J	W	S	E
---	---	---	---	---	---	---	---	---	---

D 

--	--	--	--	--	--	--	--	--	--

5 

S	B	A	K	L	I	S	N	E	H
---	---	---	---	---	---	---	---	---	---

E 

n	e			e	d			l	e
---	---	--	--	---	---	--	--	---	---

## Youth Camp

I attended last Octobers HFQ Youth Camp located at Emu Gully. This was in part thanks to the financial assistance of HFNSW.

I got to Emu Gully at 8pm. After being introduced to old friends and new, it was time for bed to get some much needed shut-eye for the busy weekend ahead.

The next morning we were greeted by our Group Co-Ordinators: Dave and Maddy. We were briefed on the themes and the ideas behind the whole camp and the individual activities. The camp was based around an ANZAC theme, and all of the activities promoted the character values of Courage, Mateship, Perseverance and Sacrifice. All of the activities incorporated either one or many of these ideals.



We did a myriad of activities and trials. My favourite of which was "The Tunnel Rats of Vietnam" where we had to crawl through concrete pipes placed under ground that twisted and turned, went back up to the surface and down. I crawled through mud on my back, climbed up a steep pipe using only my hands and knees before I had to use rock-climbing hand-holds to navigate the inside of a water tank that was knee deep in dark muddy water.

After a swim in the dam and a lovely dinner thanks to the wonderful chefs, it was time for

us to go on a night walk. Were we had to carry one of the members of our party on a stretcher the whole night, including down a giant slide, through a bunker, and a maze where we weren't allowed to talk at all, and the toughest, was up a steep hill. All of the hard work and heavy lifting was worth it for the end of the walk, because we got to ride in the back of an old Vietnam War era troop-carrying truck all of the way back to the campsite.



After some supper it was time for bed, and boy did we all sleep.

Too quickly, Sunday came, but there was a sadness around camp in the morning. This was the last day of camp, it had come too soon. All was not lost though, because when we met Dave in the morning, he prepared us for our

first activity of the day. The infamous "Mud Course". Unfortunately due to injuries sustained on the day before I was unable to actually do the course with my fellow campers, so I became the designated camera man.

The "Kokoda Mud Trail" consisted of about 40 metres of waist deep mud, mud hills, climbing under and over wooden poles and random holes that you could fall in and cover yourself even more thoroughly in mud. After a quick shower in the dam, we had

lunch and then our final activity. The Twister Buggies! After a few lightning fast laps on in the little buggies, 4pm had come, and it was time to high-tail it out of there to get to the airport on time. After some teary eyed goodbyes, we packed up the van and headed out on the road.

The weekend was over, and I was very grateful to have been a part of it.

A key component of the HFQ camp was the fact that it was very physical, kids were running around, hurting themselves (accidentally) and doing physically strenuous things with no-one telling them they cant. It lets kids be themselves away from their parents constantly hovering over them like they do at Family Camp.

[Emu Gully provides an] ANZAC focused camp, with the themes of self-sacrifice and courage [which is] a really important experience. It might also help parents sending their kids off to have fun and raise hell, to know that they are being taken care of by proper recreational educational facilitators.

Samuel Linnenbank (NSW)  
slinnenbank@gmail.com.

If you would like to attend the next youth camp or have any questions please contact the office and we'll connect you with one of the youth mentors

# PROBE Study

How can haemophilia foundations have access to good quality data about the treatment and health experiences of people with bleeding disorders? The multi-national PROBE (Patient Reported Outcomes Burdens and Experiences) Study aims to do precisely that. Haemophilia Foundation Australia has joined the PROBE investigation team to be part of this important international study on the impact of living with a bleeding disorder.

PROBE is a patient-focused research project led by a global team of patient and academic investigators, including Mark Skinner, former WFH President, and Assoc Prof Alfonso Iorio from McMaster University, Canada, who have worked closely with HFA on the Australian arm of the study. The research will support efforts to improve treatment and comprehensive care programs in Australia and other countries around the world.

## Phase 1

Some of you have been involved in distributing and completing the surveys for Phase 1 of the PROBE study. Thank you for your support! We needed 35-50 completed surveys by the end of 2015 to have enough data to be analysed, and by Christmas had received 39 surveys, which was an amazing effort in a very short few weeks!

What did Phase 1 involve?

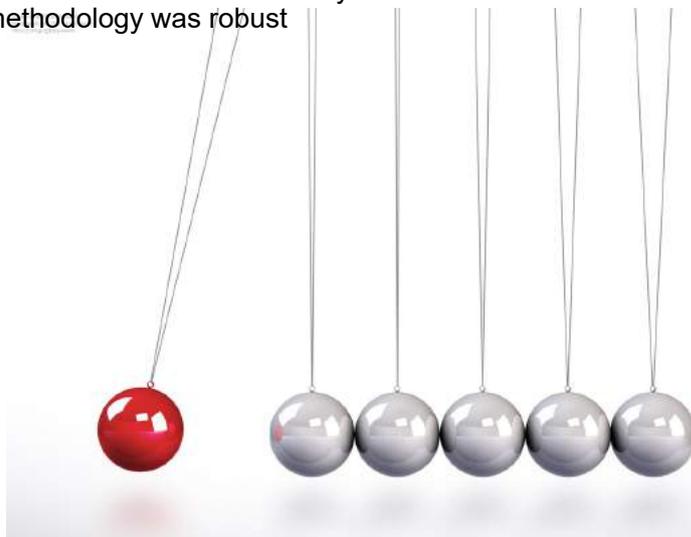
- Seeking ethics approval via Monash University, Melbourne
- Testing how clear the questions are
- Testing the methodology and the statistics that could be gained from the results.

Phase 1 tested a print survey on haemophilia only. Some Australian community members with haemophilia and carers or parents of children with haemophilia completed the draft questionnaire and gave feedback on the questions and instructions. The completed surveys and question feedback sheets were then scanned and transferred electronically to the PROBE research team at McMaster University for analysis.

HFA received a report on the Australian results in January 2016 and was invited to participate in an

international meeting where the worldwide results were discussed:

More than 700 surveys completed internationally Results demonstrated validity & that the research methodology was robust



Phase 1 data is useful for understanding how to use the potential results but should not be used for advocacy at this point – wait for phase 3

Australian feedback on questions was put in a spreadsheet and each comment addressed by the research team leaders

As a result of global feedback, new questions will be added in Phase 2 for women who carry the gene For good comparable data, in future Australia needs to recruit more people with moderate haemophilia to complete the survey.

## Phase 2

The next phase involves reproducibility – testing an online version of the survey as well, and retesting the same community (ie, Australia) to see whether the results are consistent. Stay tuned!

If you have any questions about the PROBE survey, please contact Suzanne at HFA on 1800 807 173 or [socallaghan@haemophilia.org.au](mailto:socallaghan@haemophilia.org.au); or visit <http://tinyurl.com/PROBE-Aus>.

*Update from HFA*

# Answers to puzzles in the centerfold

Word Boxes:  
 Box 1 = Box E (Needle)  
 Box 2 = Box A (factor)  
 Box 3 = Box D (vein)  
 Box 4 = Box B (infuse)  
 Box 5 = Box C (saline)

Riddle: I am Factor  
 Story Fill: haemophilia; safe; knee; swell; infusion; factor; hobbled; rest; ice; compression; swings

## What is Pain & what happens when we feel it?

Our body contains specialised nerves that send alerts to the brain about potentially dangerous changes, but they do not send pain to the brain because all pain is made by the brain. Pain can be “turned on” or “turned up” by anything that provides the brain with credible evidence that the body is in danger and needs protecting.

Pain is not actually coming from the affected area. Pain is the result of the brain evaluating information, including danger data from its danger detection system, cognitive data such as expectations, previous exposure, social beliefs, and other sensory data such as what you see, hear and sense.

The brain produces pain; it uses all the incoming data and stored information to identify the source location. Usually the brain gets it right, but sometimes it doesn't. An example is referred pain in your leg when it is your back that might need the protecting.

It is pain that tells us not to do things – for example, not to lift with an injured hand, or not to walk with an injured foot. It is pain, too, that tells us to do things – see a physio, visit a GP, sit still and rest.

It might seem that pain is all about the brain and not about the body at all but this is not true either. “Danger detectors” are distributed across almost all of our body tissues and act as the eyes of the brain.

When there is a sudden change in tissue environment – for example, it heats up, is squashed, squeezed, pulled or pinched – these danger detectors alert the brain and mobilise inflammatory mechanisms that increase blood flow and cause the release of healing molecules from nearby tissue, thus triggering the repair process.

Local anaesthetic renders these danger detectors useless, so danger messages are not triggered. As such, we can be pain-free despite major tissue trauma, such as being cut into for an operation.

Inflammation, on the other hand, renders these danger detectors more sensitive, so they respond to situations that are not actually dangerous. For example, when you



move an inflamed joint, it hurts a long way before the tissues of the joint are actually stressed. Danger messages are highly processed along the way to the brain. The danger transmission neurones that run up the spinal cord increase and decrease their sensitivity according to what the brain suggests would be helpful.

So, if the brain's evaluation of all available information leads it to conclude that things are truly dangerous, then the danger transmission system becomes more sensitive. If the brain concludes things are not truly dangerous, then the danger transmission system becomes less sensitive.

Danger evaluation in the brain is mind bogglingly complex. To understand how pain works in real-life people with real-life pain, we can apply a reasonably easy principle: any credible evidence

that the body is in danger and protective behaviour would be helpful will increase the likelihood and intensity of pain. Any credible evidence that the body is safe will decrease the likelihood and intensity of pain. It is as simple and as difficult as that.

### Implications

To reduce pain, we need to reduce credible evidence of danger and increase credible evidence of safety. Danger detectors can be turned off by local anaesthetic, and we can also stimulate the body's own danger-reduction pathways and mechanisms. This can be done by anything that is associated with safety – most obviously accurate understanding of how pain really works, exercise, active coping strategies, safe people and places.

A very effective way to reduce pain is to make something else seem more important to the brain – this is called distraction. Only being unconscious or dead provide greater pain relief than distraction.

With some chronic pain the relationship between pain and the true need for protection becomes distorted: we become over-protected by pain. This is one significant reason there is no quick fix for persistent pains. Recovery requires a journey of patience, persistence, courage and good coaching. The best interventions focus on slowly training our body and brain to be less protective.

*By Prof Lorimer Moseley. University of South Australia. Prof Moseley spoke at our conference in October. This article was published in [www.theconversation.com](http://www.theconversation.com) on 19 November 2015*

**Ed - The next Life Series Workshop is focusing on Managing Chronic Pain. If you would like to attend or want more information please call the Psyc/social workers at RBWH.**

**Loretta: 07 3646 8769**

**Mona: 07 3646 7937**

## My ABDR Update

### WHAT'S NEW?

An update to the MyABDR web version was released in October 2015, bringing you more improvements and features including:

Ability for users to request their own updated emergency patient card and whether they would like it sent to their Haemophilia Treatment Centre or directly to their own nominated delivery address – click on the Details button.

Please note: all card requests will always be checked by staff at your Haemophilia Treatment Centre.

The next release is scheduled for January 2016 and will be a release specifically for the smartphone app looking at how, when and why the app syncs and doing a complete review to enhance the process of saving and updating your data.

Make sure you update your MyABDR app to take advantage of these new enhancements and fixes. These improvements are

always developed in response to user feedback so please call the HELP number below to tell us about how the app works for you.

If you need some help with getting started, there is also a new quick reference guide: 6 easy steps for recording treatment with the MyABDR app.

Download it from the web site – [www.blood.gov.au/myabdr](http://www.blood.gov.au/myabdr)  
Or ask Suzanne at HFA to send you a copy – [socallaghan@haemophilia.org.au](mailto:socallaghan@haemophilia.org.au);  
ph 1800 807 173

### MyABDR AT THE HFA CONFERENCE

MyABDR was the focus of the National Blood Authority booth in the exhibition at the 2015 Gold Coast Conference.

The MyABDR support team enjoyed meeting with new and existing users to fill you in on the latest developments and what is planned, get your feedback and suggestions, and to help you with any queries. If you are interested

in joining the focus group all are welcome please register your interest with Suzanne at HFA.

### MYABDR FOCUS GROUP

The Conference was also an opportunity to have a session with some of the MyABDR Focus Group members. Thank you to the participants who gave feedback on proposed enhancements to MyABDR and worked through ideas on future development. This was very helpful!

### NEED HELP?

Don't hesitate to contact the MyABDR Support team with any queries. Meghan, Danny, Lachlan or Rebecca are always available and happy to assist you.

T: 13 000 BLOOD / 13 000 25663  
E: [myabdr@blood.gov.au](mailto:myabdr@blood.gov.au)  
Available 24 hrs a day, 7 days a week.

From the HFA and National Blood Authority  
MyABDR Team

## Six easy steps for recording treatment



1. Download it free from the Apple **App Store**, or **Google Play**
2. **It's easier to register on the web site!** Go to <https://www.myabdr.blood.gov.au> and click on **New user?** or **Register on the app**. Open it and tap **CREATE AN ACCOUNT**. Then Complete the registration form.
3. When you receive your password, open the app and login to MyABDR. Change your temporary password to a password of your choice.
4. **Important! - Add your treatment product stock to your inventory first before recording a treatment.** Tap the **RECORD PRODUCT** shortcut on the home screen (Only the treatment products that are in your treatment plan will be shown) then enter product details and tap **DONE** when finished.
5. Tap the **RECORD TREATMENT** shortcut on the home screen.  
**What type of treatment?**  
The information button has a list of the treatment definitions – Enter treatment details and tap **DONE** when finished.
6. You can record bleed details when you are recording a treatment for the bleed. In the **RECORD TREATMENT** screen; tap in the **TREATMENT TYPE** box and select **BLEED** from the pop-up list.

## The value of recording our experiences.

As we continue to face increased scrutiny over health care costs, funders have started to demand evidence based treatments before continued funding is approved. One difficulty is that research data does not always take into consideration real-life treatment experience and the significant quality of life improvements that treatment regimens, such as prophylaxis has provided for people with bleeding disorders.

Past research has shown a clear correlation between the severity of haemophilia and the age bleedings started as well as the number of joint bleeds. Doctors used this data to conclude that regular infusions are the best treatment regimen for patients who have the most frequent bleeds.

For the community with bleeding disorders here in Queensland, it is inconceivable that this basic treatment regimen might need additional proof through randomised controlled trials (RCT's). However, this discussion is beginning to be raised in some countries and may happen here.

We live in the era of evidence based treatments and the need to collect data to show the correlation between cost of treatment and our health outcomes could be avoided if we use established data bases. Within the area of haemophilia care, none is better defined than the ABDR and its patient equivalent MyABDR which collects data from diagnosis to the provision of treatment and it's use at home.

Using MyABDR is a simple way we can contribute to data collection and help ourselves. The initial benefit of MyABDR is when treatment use (recorded by the patient) is linked to treatment purchases Australia wide and this can result in cost savings.

The system knows what is purchased and distributed from hospitals and it should know what is used in your own home. This

allows precise stock control and helps prevent wastage. If we use it well ABDR could be able to ask you to use any older stock you have. This may be just one vial a year, but even one vial per patient per year on a Queensland level could pay for a significant portion of HFQ's annual funding.

Data collection didn't start with MyABDR, the government is doing its best to get everyone onto Personally Controlled Electronic Health Records (now called 'My Health Record') and you are probably on several other databases already, like the mail list for this magazine.

With 'My Health Record' medical treatments can linked to your unique patient identifier. In parallel, the ABDR registry records your bleeding disorder history.

The advantage of MyABDR is that it is part of the ABDR and the information captured expands as bleeding incidents, and treatment occur. Entering incident and use data may feel like a hassle but we believe it is an investment in the future development of haemophilia care and monitoring of treatment and its outcomes for everyone in our community.

If we use it well the advantage will be timely access to specific information on the people for whom we and the QHC are advocating and the more that goes into MyABDR the better! Without data, patient organisations like ours are limited to fighting with just emotional arguments, which for most governments is not sufficient.

In the future MyABDR may be used to identify annual trends—number of new patients, approximate bleed rates, etc. But right now it also lets you remember what happens to you between appointments and your treatment can be better tailored to your individual needs.

If we use it well the days of filling out treatment sheets before an

appointment from memory will be over. Instead, you can use MyABDR to enter data on bleeds, product use, timing, and pain experienced during or close to treatment administration. Not only is this more accurate, but it prevents vague discussions at your QHC appointments.

Going forward the capabilities of MyABDR will continue to be enhanced. This ensures that there will be increased value derived from the data that is collected.

It might be able to track and warn patients approaching their factor trough levels or alert the QHC when you enter a persistent or specific bleed into MyABDR.

MyABDR might also assist with identifying a bleed. In future it may be able to use accelerometers in smartphones to detect changes in gait, which may be indicative of the start of a bleed.

Good use of MyABDR by our community could avoid any attempt at narrow focused RCT measurements that might be used to determine who gets treatments in the future.

It is our responsibility not to place future PWBD are not faced with situations where their quality of life could be put at risk because the increased focus on financial analyses.

There were many clinicians and community members willing to contribute to the development of MyABDR but we now need everyone in Queensland using blood products to collect and input the data so that future problems can be avoided we can learn from the data gathered.

*Inspired by two recent articles in Hemophilia World (<http://www.hemophiliaworld.org/>) written by: Marijke van den Berg, called "Historical perspective on the treatment of hemophilia"; and by Declan Noone, in Hemophilia World called "Electronic patient records and databases"*

## Hep C drugs go onto PBS

It was a very welcome Christmas message for our community: on 22 December 2015 Australian Minister for Health Sussan Ley announced that new breakthrough hepatitis C treatments will be available on the PBS from 1 March 2016.

and affordable to all Australians with hepatitis C.”

Most of these treatments can be taken orally, with the most common course of treatment being as short as 12 weeks.

disorders can access treatment when needed in a timely manner,” noted Gavin Finkelstein. “For our community members, the message about being proactive with your hepatitis C care is even more important now: look after your

liver health, make sure you have had your liver health checked and talk to your hepatitis C clinic about your treatment options.”

Read Minister Ley's Press Release – <http://tinyurl.com/hepctreatments-dec15>

### AND IF YOU HAVE HEP C?

In the meantime, if you have hepatitis C and a bleeding disorder, remember that you would need to have your liver health assessed before you could be considered for

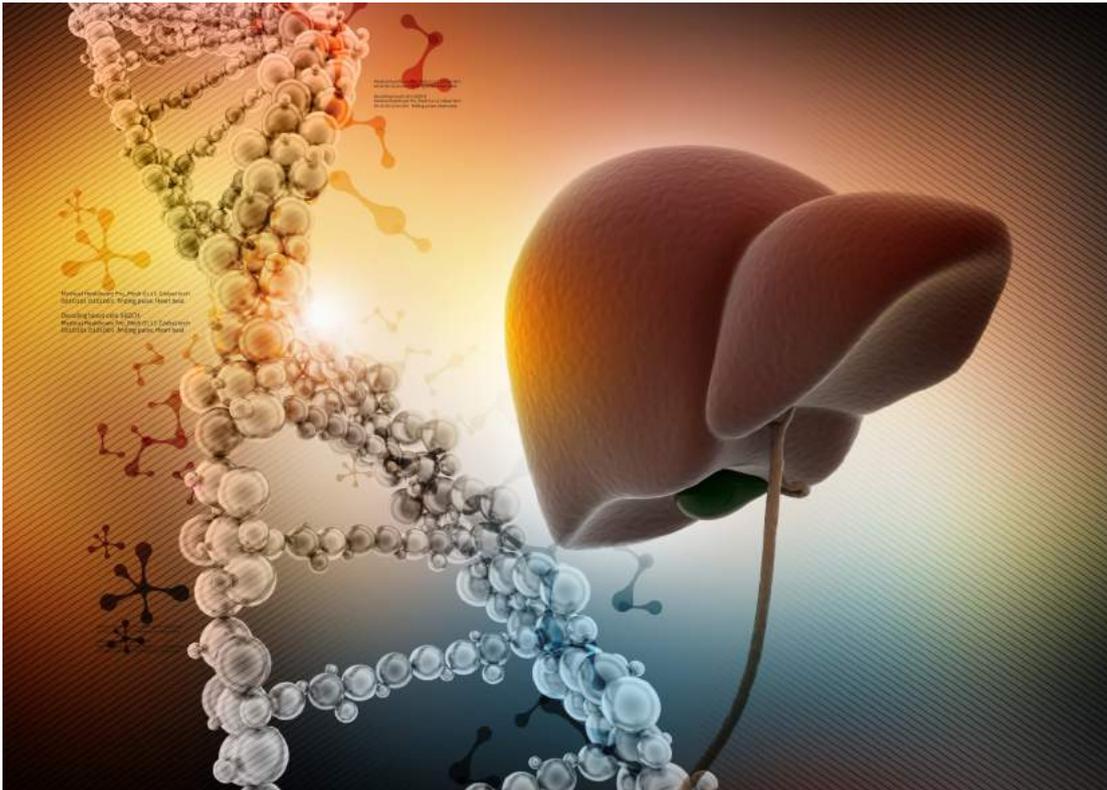
treatment – don't wait; if you haven't already, make your appointment now!

Make sure you have your liver health checked regularly. If you don't know where to start, ask your Haemophilia Centre for a referral.

Stay in touch with your hepatitis clinic about what's new. Don't forget to go to your appointment with the hepatitis clinic after your liver health check, even if the fibroscan shows your liver health is stable at the moment.

And for comprehensive care, let your Haemophilia Centre know about your liver test results or how your treatment is going to make sure they stay in the loop.

*Update provided by HFA*



These medicines are:

- sofosbuvir with ledipasvir (Harvoni®)
- sofosbuvir (Sovaldi®)
- daclatasvir (Daklinza®)
- ribavirin (Ibavyr®).

“This is fantastic news for people with bleeding disorders and hepatitis C,” said Gavin Finkelstein, President of Haemophilia Foundation Australia. “They have been waiting so long for access to treatment to cure their hepatitis C. Many have seen their liver disease progressing and were despairing. This decision by the Government will change people's lives and we would like to congratulate Minister Ley for seeing the process through to make these treatments available

“This combination of breakthrough cures has a success rate of more than 90 per cent across the entire hep C patient population and is faster and has fewer side effects than anything currently available,” said Minister Ley.

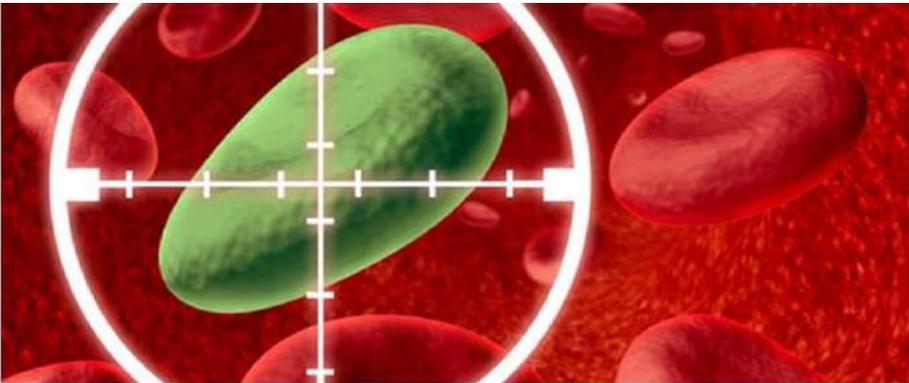
Listing the new medicines on the PBS will mean that people with hepatitis C will only pay the normal PBS co-payment for these treatments: currently \$6.10 for concessional patients and \$37.70 for general patients each time the medicines are dispensed by the pharmacy.

“HFA will continue to work with expert health professionals and health services to make sure that people with bleeding

# CRISPR

Scientists have been tinkering with genes since the early 1970s to create faster growing wheat or herbicide-resistant crops and for the last decade or so gene therapy treatment has been “about to cure” haemophilia according to the experts.

Until now the techniques had poor precision and it required tens of thousands of individual gene that had to be tinkered with to achieve the required edits and usually those edits would be inserted on the wrong pages of the DNA text.



The news from last year was a big advance called CRISPR technology. It has created a lot of interest and headlines, due to its cheap price and ease of use. When that translates to therapies available for ‘everyday’ use and which bleeding disorders it will work on are still not answered.

To reliably manipulate specific genes to fight human diseases or targeting inherited conditions, pinpoint precision has been required.

This is what the CRISPR gene editing technique offers. Since scientists first discovered CRISPR in bacteria three years ago, it has taken the world by storm. Microbes evolved CRISPR to edit viruses out of their DNA. Now it’s been used outside the body to precisely edit everything from the DNA of crops to editing the HIV virus out of human DNA. Editing and tampering with the DNA of an embryo or inside patients has been considered out of bounds.

Gene editing refers to the process

of deleting, adding or altering DNA in precise spots in a genome. CRISPR is a cheap, easy and has the potential to treat numerous diseases. It has not yet been tried in people but older, more expensive forms of gene editing have already been used in cases of leukaemia and HIV infection – although cells have been removed from the body first for their genes to be edited.

The challenge is delivering the CRISPR machinery into T cells. Once there, CRISPR itself can

then act like a guided missile, homing in on a precise stretch of the DNA code.

The first attempt to edit the genes of cells inside the human body is about to take place. The trial will use an older technique, based on a DNA-cutting tool – called a zinc finger nuclease – that can be injected straight into the bloodstream to do its work.

### Viral delivery

But now it may be possible to insert a corrected version of the mutated gene into the genome, using the DNA-cutting tool which uses a (harmless) virus to deliver it to the liver with a missing Factor IX gene. This has been successfully done in mice and primate trials already.

The researchers designed the tool to insert the Factor IX gene next to a promoter for a gene coding for albumin, a major constituent of blood. A promoter is a bit like a volume knob – it regulates the activity of nearby genes. Placing the Factor IX gene

nearby means the liver starts producing lots of clotting protein.

The treated mice and primates started producing regular levels of Factor IX, which caused their blood to clot as normal. The team will now trial the technique in adults with haemophilia B.

### Keeping on target

One of the major concerns with all types of gene editing is off-target effects: the possibility that the DNA-cutting enzyme will make cuts in unwanted places. This has resulted in several scientific conference discussions in the last twelve months.

There are very serious concerns about potential off-target effects in the upcoming haemophilia trial because the treatment is permanent. Over time, any nuclease would eventually bind and cut an off-target site.

Sangamo, the biotech company that developed the DNA-cutting enzyme say it only has one off-target effect, and this is in a gene called *Smchd1*, which produces a protein that helps turn other genes on and off. They were required to do a whole bunch of assays to prove to the US Food and Drug Administration that their procedure meets safety requirements.

Should this approach prove effective, Scientists will be able to use it as a treatment for other conditions that involve a missing protein. We understand there will be a lot of updates on this trail and other uses of CRISPR at the World Congress of the WFH in Orlando this July and we’ll report those findings once we have them.

*H Factor editor. Adapted from various sources and updates. For more information check out a great articles in New Scientist at: <https://www.newscientist.com/article/mg22830522-200-crispr-will-make-2016-the-year-of-gene-edited-organisms/> And <https://www.newscientist.com/article/dn28619-first-trial-of-gene-editing-treatment-for-haemophilia/>*

# Health Updates

## Cuts to Pathology payments should not impact on patients

In last years Mid-Year Economic and Fiscal Outlook (MYEFO) the government flagged changes to abolish the bulk-billing incentive for pathology tests. There were fears that patients could be charged significant out-of-pocket pathology co-payments for tests for blood, tissue and other bodily fluids.

This will NOT happen to hospital tests and may not happen at all because consumers almost never initiate pathology services. Doctors order tests to assist them to make a diagnosis or to track a patient's condition so there is no theoretical argument to use co-payments to limit demand.

Technological advances and company amalgamations have also happened over recent decades. The two big pathology companies do 75% of all Medicare-billed tests and many parts of the sector are now automated. The large companies benefit from economies of scale as the costs of an additional test to run through an analyser are trivial.

Just as the ABA tenders for the best price with factor and other bleeding disorder treatment products, the government could go to tender for the right to bill Medicare for pathology.

Tendering should generate savings of 10-20% compared to the current Medicare costs. This happens in Victoria who say tendering has introduced price competition into their pathology market.

There are savings to be made in pathology payments so don't worry next time you see your doctor and they request a blood test.

*Graham*

## Zika Virus Update

Zika virus has been declared a public health emergency. The virus have now been reported in Queensland in people who travelled from affected countries such as Brazil and the World Health Organisation estimate that up to 4 million people worldwide could be affected this year.

Zika is primarily spread by mosquitoes. Zika is a relatively large virus and there is a high probability that it will be successfully inactivated and removed by current viral inactivation and removal techniques used in the manufacture of plasma-derived medicinal products, including plasma-derived FVIII and FIX concentrates.

There have been some anecdotal reports of transmission by blood transfusion and via sexual contact but transmission via blood supplies is more of a potential issue. So, blood authorities may consider a temporary deferral from blood donation of persons with a travel history to affected areas, as used for dengue fever).

60-80% of infected people have no symptoms, and the majority of symptomatic people have mild symptoms, including fever with muscle ache, rash, and red eyes.

The major concern is the possibility of infected pregnant women passing on microcephaly to their unborn babies. There has also been an increase in the number of cases of Guillain-Barre syndrome probably related to an immune response to the virus.

The World Federation of Haemophilia (WFH) will update us when more information is available. See also: <http://www.wfh.org/en/our-work/treatment-safety/zika-virus-communicue?erid=795059&trid=3d29ef5b-4dec-4b7c-ad64-aef61866bcd6>

## Fundraising campaign started for haemophiliac puppy Bailey

Anyone who saw him would never guess there was anything wrong.

Just like a normal dog, Bailey wags his tail, chases sticks and goes for walks. But he isn't a normal dog. The 18-month-old pup has haemophilia A, meaning that regular doggy activities could be fatal (or costly).

He was diagnosed at just 14 weeks, when his owners took him to the vet for a microchipping and the vets quickly became worried after the canine started bleeding from the small wound soon after.

Bailey faced an uncertain future, with the congenital disease not covered by his insurance and his owners unable to pay the AUS\$3,000 bill for his first treatment – let alone the AUS\$1,5000 predicted cost of any future incidents.

Fortunately, the practice stepped in and their head nurse gave him a new home.

She said: "When he first came in he was like a little cloud - you couldn't really say no to him and I felt like I had the resources to provide an ok life for him.

"He comes to work every day because I can't leave him on his own in case he hurts himself. "He lives in the office, with some other friends, and we've learned a lot from having him around because it's a condition we've never managed as a group of vets before and we've got a lot of staff who had never even seen it before he came along.

For Bailys story see: <http://www.maidenhead-advertiser.co.uk/News/Areas/Maidenhead/Fundraising-campaign-started-for-haemophiliac-puppy-22022016.htm>

## Important Dates for HFQ Members

**Bec's Booster Class** First Tuesday of the month

**World Haemophilia Day** 17 April 2016.

**Wonky's** (Previously OBEs) An informal support group for men living with a bleeding disorder. Meets in SE Queensland on the first Wednesday of each month.

**Regional Meetings** The Haemophilia Clinics travel to centres with clusters of people with bleeding disorders. Where possible HFQ try to host a local meeting to coincide with these visits so please ask if one is happening at the time of your next centre visits. More details will be in later issues.

**Haemophilia Awareness Week & Red Cake Day** 11 – 17 October 2016

**HFQ Youth Camp** 28 – 30 October 2016

Please call Graham at the office on **07 3017 1778** for more info on any of these events and activities.

## Go For It Grant Winners



### The HFA Go For It Grants for 2015-2016 have been finalised!

The Haemophilia Foundation Australia's Go for it Grants program were offering 2 x \$5,000 (supported by Pfizer) to provide winners with the financial support to help pursue their dreams. HFA recently announced that the panel of judges has awarded three grants rather than two as initially advertised. The Grant recipients were: Sam Linnenbank of NSW; Ian Zaro from Qld and a third provisional grant that will be announced in due course.

Sam Linnenbank was awarded his to attend the WFH 2016 World Congress as part of learning more about community representation and leadership and we congratulate him.

Ian Zaro received a grant for recording equipment to support his passion for comedy and further develop his communication skills for social media. We are of course excited for Ian as an HFQ member and strong youth peer mentor and advocate. Ian is already creating his own Dub-Smash clips and starring in the ABC comedy series Black Comedy. We look forward to seeing what Ian does in future.

### STOP PRESS

The Damien Courtney Memorial Endowment Fund has now opened for anyone with a bleeding disorder or affected by bleeding disorders in Australia to apply for projects, services and/or care aimed at improving their physical and emotional wellbeing and independence.

The fund has \$15,000 available to disburse and applications close on 20 May 2016. Please contact HFA via their website, or on 1800 807 173 for more information.

## About The 'H' Factor

The 'H' Factor is published four times each year by HFQ. We occasionally send important information and updates on local and relevant events for people affected by bleeding disorders to subscribers of our email list. If you would like to be on the HFQ Email List, please register your interest by sending through an email with the subject title 'The 'H' Factor email list' to [info@hfq.org.au](mailto:info@hfq.org.au). You can be removed from the list at anytime.

**Graham Norton**  
HFQ Manager & The 'H' Factor editor  
Ph: (07) 3017 1778 E: [info@hfq.org.au](mailto:info@hfq.org.au)