the H FACTOR

Issue 45

Summer 2015

Newsletter of Haemophilia Foundation Queensland

From the President

Hello everyone,

This month I would like to touch on the topic of inhibitors for previously untreated patients (PUPs). The world federation of haemophilia have recently asked the US Food & Drug Administration (FDA) and the European Medicine Agency (EMA) to examine all relevant data and come to a conclusion about recent outcomes from studies. This comes as a result of a recent study published by a group in France suggesting recombinant factor VIII product is associated with a higher risk of inhibitor development in newly diagnosed, previously untreated patients with severe haemophilia A.

The group in France suggested a higher incidence than expected and follow on from the unexpected results from the RODIN study (Jan 2013) which was reviewed by regulators in Dec 2013 by EMA & the committee on human medicinal products – they endorsed recommendations which concluded that the benefits of Kogenate FS/Bayer/Helixate Nexgen continue to outweigh the risks in PUPs with haemophilia A. The EMA stated that the product information for this product should be amended to reflect the results of the RODIN study and clarify there is no different risk between products.

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Inside this issue:

Presidents Message *Continued...*

Inhibitor development is caused by many risk factors, which make it different to make conclusions in a small patient population. At the moment, no firm conclusions can be made.

Both the FDA and the EMA have confirmed they will re-examine the data. In Jan 2015 EMA confirmed it will work with the investigators of all the relevant studies regarding Kogenate and inhibitor development in PUP's – the review is expected to take several months. It is the view of WFH that all of the available data should be pooled in order to give a clearer answer about the relative risk for individual products. WFH goes further recommending it is prudent to consider not using Kogenate for newly diagnosed PUP's with severe haemophilia A where other safe clotting concentrates are available. Note – there is currently no known increased risk for any other patients using these products.

WFH are monitoring the situation and will communicate relevant information when available

Remember, if you have any concerns or questions, please familiarise with the facts and contact your haemophilia treatment centre.

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

Note: *to read the WFH article please go to page 16* ⟶

Farewell to Maureen

Some of you may not know that our lovely, honourable Advanced Social Worker Maureen Spilsbury has decided to retire after 18 years at The Queensland Haemophilia Centre.

As I can't speak for everyone, I'm sure that most of you will agree with me that Maureen is going to be missed a lot (a bit like our clotting factor/s).

Maureen really knew how to place a smile on your face or make you laugh even when your pain score was 10/10.

Maureen was there for you when you needed her, if it was just to talk, listen, help you out with a referral letter/s to help with getting work, grants, travel cost, accommodation etc.

Maureen also gave you advice (even if you didn't want to hear it, which going by my own experiences; all Maureen was doing was her job and was always right in the end), no matter what it was Maureen could help you with a problem and if she couldn't help you, Maureen would know someone who could.

Maureen knew every patient at The Queensland Haemophilia Centre and was highly respected by people with a bleeding disorders, parents and work colleagues (not just in the QHC but also throughout Royal Brisbane & Women's Hospital, regional parts of Queensland and Australia/New Zealand wide).

On a personal note, if it wasn't for Maureen who made me decide on a trial (which Dr Rowell wanted me to do and that was to use 4000 units of Advate 3 days a week to try and get rid of my Inhibitor); I wouldn't be able to report that since starting the trial back in April 2015, today my Inhibitors levels are negative and I haven't had a bleed since then either.

On behalf of everyone that had something to do with Maureen, I would like to thank Maureen for her kindness, caring nature, warmest touch but most of all the love that she gave to everyone in the last 18 years. No doubt Maureen is going to be missed and extremely hard to replace and I'm sure everyone would like to wish Maureen all the best in the future.

Written by Brett Williams on behalf of all of us at HFQ

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) 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* or at our Facebook page at *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*

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
Desdemona (Mona) Chong – Advanced Psychologist (Fridays)	3646-8769

Advanced Social Worker (tba)

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.

Managing Change

Change — noun, an act or process through which something becomes different.

A new year beckons. New year resolutions await. New year resolutions are really another way of saying "I need to change something I am currently not happy with". Such changes are deliberate. However, change can also happen when we are least expecting it. Most times, we have no control over such changes as well!

Change happens in the journey of the haemophilia community as well. Baby grows older and needs to have a port insertion.

⇒ From a port insertion to venipuncture.

⇒ From venipuncture to self-infusion.

⇒ From paediatric to adult centre. From singlehood to parenthood.

The list goes on.

How can we better navigate these changes?

1. Recognise the worst
Understanding a change is the important first step to managing it. We're usually scared of change because we're afraid of the unknown. Try to figure out if the worst that can happen is actually as bad as what you think can happen? Find out more about what the change actually involves. Remind yourself that things are often not as bad as you imagine it to be.

2. Focus on what you can control
When a big change happens, figure out what aspects you can control and what you can't.

3. Take action
If the unwanted change is within your control, taking an active approach to coping has been shown to be useful. This means doing something about the situation e.g. problem-solving, developing a plan of action, seeking help or goal-setting.

4. Accept and reframe
If the unwanted change is beyond your control, taking a reflective approach may be the way to go. Accepting that there are things beyond your control and choosing to be comfortable with this is likely to bring greater calm than playing the blame game or waging unwinnable wars. This is where the famous Serenity Prayer may come in handy as a reminder.

5. Paradoxical gratitude
This involves being grateful for the situation (or change) you rather not have in your life. For example, if you are stuck in traffic, it helps to remind yourself that you are stuck because you can afford a car or being stuck in traffic is better than being in an accident. Finding something to be grateful about might not come easy and it might also take some time, but it's worth it.

6. Manage your stress
Improving your ability to handle stress will go a long way to helping you deal with change. Try some mindfulness or meditation or other activities that help to calm you down. Simple techniques can help to regulate your emotions on a daily basis which means you can think better, make better decisions and stay healthy. Check out the Resilience Workshop advertisement in this newsletter!

7. Seek support
A little support goes a long way, whether it is practical support (e.g. muscle power to move house), or emotional support (e.g. someone to listen to you). You can consider approaching your family or friends as a start. Peer support seems to be extremely valuable within the haemophilia community. There would usually be someone who has taken a similar road to the one you are travelling and can offer some tips. Otherwise, you can seek professional help through your GP, local psychologist, help-lines or the Foundation.

By Mona Chong.

Article adapted from http://au.reachout.com/7-tips-for-dealing-with-change

Changes in Nursing Staff at RBWH

We are saddened to report the departure of Olivia Hollingdrake, Clinical Nurse at the Royal Brisbane and Women's Hospital Haemophilia Centre but very pleased to welcome Alex Connolly in her stead. We've asked each to have a few words...

Farewell from Olivia

At the end of October I left my position as Clinical Nurse Haemophilia after seven years. I have been offered a three year scholarship to study for my PhD in HIV social research at the University of Queensland. While I am very excited to pursue this new opportunity, I am already missing many of the resilient and inspiring people that I met whilst working at the QHC. The skills and knowledge I gained from working with the bleeding disorders community will be used again and again in my new role, and I imagine in many roles to come. I know you will make Alex very welcome as she takes over the clinical nurse position.

Olivia Hollingdrake

Greetings from Alex

Thank you Graham for the opportunity to introduce myself to the HFQ

I'm the newly appointed clinical nurse for the Queensland Haemophilia Centre. I have been working at the Royal Brisbane and Women's Hospital since 1998. My previous experience has been as a nurse working on a general surgical ward and for the last ten years as a clinical nurse within the peri-operative division. I have had some experience with looking after patients with Haemophilia and other bleeding disorders and this always created a great interest for me.

Recently I had the pleasure and privilege to attend the recent Australian & New Zealand Haemophilia Conference on the Gold Coast. It was a new experience to attend a conference where there was a great representation of patients as well as health care professionals and pharmaceutical representatives. I gained a lot of knowledge about comprehensive care, new treatments for Hepatitis C, mindfulness and various other very interesting topics. The conference was a great way to start a new part of my professional life!

I am really looking forward to working at the Haemophilia Centre. And remember we are just a phone call away!

Alex Connolly

Quality of Life Study is Underway

Thank you very much for those who have completed the questionnaire pack. We have about 30 responses so far. Most participants have been approached through clinic visits.

We would love to collect as many responses as possible in order to get a good representative of the community and am inviting everyone with either Haemophilia A or B to participate in this study.

If you are able to contribute to this study (takes 30mins to complete a questionnaire pack) or find out more before you decide, please contact Mona *(email: desdemona.chong@health.qld.gov.au Phone: (07) 3646 8769 — please leave a message).*

Invitation

Breeze Life Coaching

YOU ARE INVITED TO THE

RESILIENCE WORKSHOP

OF THE QUEENSLAND HAEMOPHILIA CENTRE

FRIDAY 4 MARCH 2016

Resilience is the ability to properly adapt and bounce back after a difficult experience. Living with a bleeding disorder means you often have to face challenging situations in your life. From a young age you learn you do not have control over many aspects of your life. This can lead to distressing emotions, disrupting your life even more.

However you can gain control by influencing how you perceive what happens to you. This can help you increase acceptance of your emotional experience. You can train yourself to do this, for example through Mindfulness exercises, which allows you to be able to identify, tolerate and reduce the impact of difficult, painful thoughts, feelings and sensations.

Having a bleeding disorder also affects those around you and partners are often the main source of support, therefore we hope that they will come along and join in. This workshop will teach you the basic principles of Mindfulness, combined with a variety of practical exercises, thinking outside the box, such as creative methods using art to become more aware of yourself in the moment.

- ARE YOU AWARE OF YOUR CURRENT COPING STYLES?
- WOULD YOU LIKE TO LEARN MORE ABOUT MINDFULNESS?
- WOULD YOU LIKE TO HAVE AN INSPIRATIONAL DAY WITH OTHERS THAT KNOW AND UNDERSTAND WHAT YOU ARE GOING THROUGH?

This workshop is brought to you by Dr Ira van der Steenstraten, MD and Life Coach at Breeze Life Coaching (www.breezelifecoaching.com). She is trained as a psychiatrist, psychotherapist and family therapist in The Netherlands and has combined this professional knowledge with her personal experience as a woman living with severe haemophilia herself. The art exercises will be co-hosted by Priscilla Jean, visual artist and art tutor (http://priscillasgallery/).

PLACES ARE LIMITED. The workshop will be held in a convenient location in Brisbane (venue to be confirmed) and scheduled to start at 9.30am.

PLEASE REGISTER YOUR INTEREST WITH MONA (*desdemona.chong@health.qld.gov.au* or phone (07) 3646 8769 - please leave a message); or let Graham know at the HFQ Office (email: info@hfq.org.au or Mobile: 0419 706 056).

REGIONAL & RURAL PATIENTS including Northern NSW are STRONGLY ENCOURAGED TO ATTEND. Funding for travel can be arranged.

This workshop is part of the Life Series Workshop which are funded by a Changing Possibilities in Haemophilia® Grant by NovoNordisk.

A Farewell from Maureen

Firstly a big thank-you to the HFQ for the opportunity to say farewell to you all through this newsletter. 2015 has been a tough year for many of us and recently I made the difficult decision to resign after 18 years in the social work role at the Centre. I am going to take some time out to simply "smell the daisies" and enjoy spending time with my family and friends and beautiful grandkids.

At the same time, I am excited that a new worker will bring freshness, energy and vigour to the role. I have many fond memories of my time at the Centre and will miss the fact that every day bought new learning experiences. I have been amazed at the physical and emotional tenaciousness of community members despite the obvious struggles. I have been delighted to have the chance to watch a number of boys who were babies when I started in the role, grow into adults and become parents themselves. I want to use this opportunity to thank you for the opportunity to learn so much from those who are part of the community both collectively and individually and also to apologise where I have not been able to meet your needs in a particular situation.

I need to also take the time to thank the HFQ and in particular Graham Norton, who has been a wonderful support for the work of the Centre over recent years. I believe that the Foundation is currently as strong as it has ever been. Graham has a delightful and enthusiastic approach to his work and has a supportive yet professional manner in all he does. If you haven't been involved with the work of the Foundation, I encourage you to give him a call. The group can only remain relevant and represent your needs with your input and the organisation is only as strong as the sum of members of the community who are connected to the group.

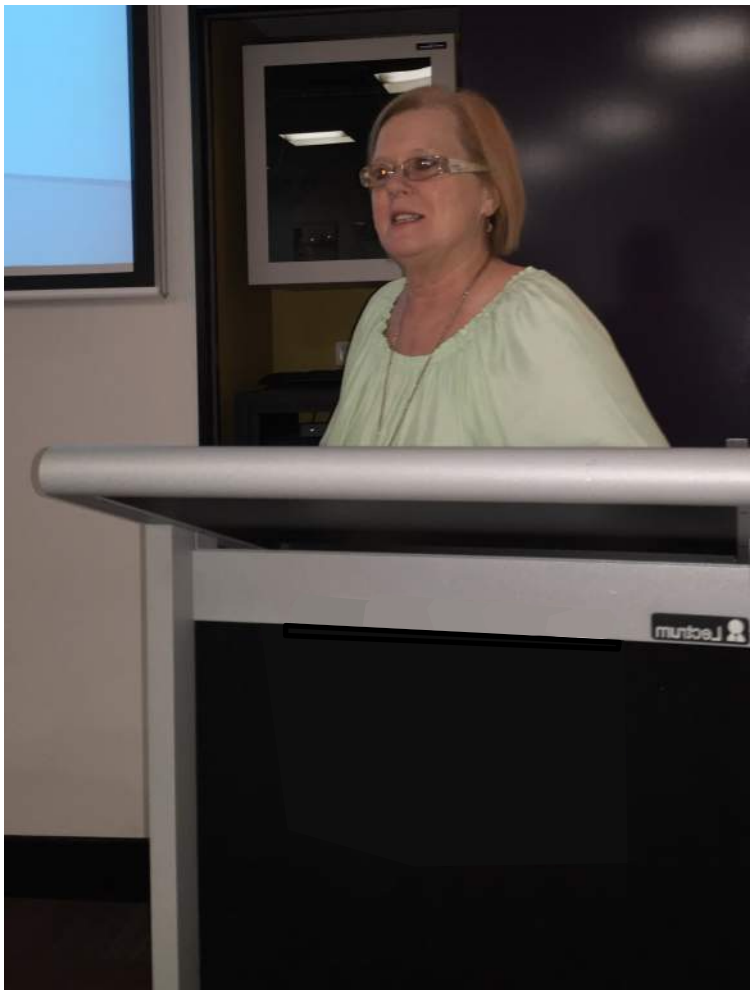
While you may not be able to, or want to attend HFQ functions there will always be other ways of offering support. Your unique skills, experience and support may foster the HFQ's continued existence into a future which is going to be plagued with increased competition for limited community health funding. The peer support offered by the HFQ is irreplaceable and NOW is the time to ensure that their support, which was borne out of individual pain and struggle, is there for your support into the future.

What can you offer? Not everyone wants to sit on committees or be involved in group activities but even a phone call to the office and a simple chat with Graham is invaluable with regards to "connection with members" for funding reporting purposes and helps update the HFQ as far as trends in community issues. There may also be ways in which you or your family and friends could help in a "one off" situation or in your local area. Louisa May Alcott who wrote the book Little Women said that "It takes two flints to light a fire" and Henry Ford wrote "If everyone is moving forward together, then success takes care of itself." Any community is only as strong as the commitment of its members. I wish each if you and the HFQ a long and hardy lifetime.

Finally I would like to thank the HFQ for the gracious gift of Life Membership of the organisation. It was an unexpected honour and will give me a reason to maintain a keen interest in the community into the future.

With fondest wishes

Maureen Spilsbury



Focus on Joint Pain Research

The hunt is on for ways to diagnose and treat the joint problems that are now the main chronic problem in haemophilia

Many of our members will be familiar with or may have bulging, painful knees and elbows caused by bleeding into their joints. Thanks to the advances in treatment, people with bleeding disorders are living longer, but arthritis and disability is an unfortunate consequence of longer life and this is driving a lot of new research.

Up till now doctors have treated synovitis by removing the inflamed tissue surgically or injecting radioisotopes into the joint to destroy the cells in the joint lining and prevent further bleeding. Such surgery is a delicate procedure and the deformities can be much more severe than someone with arthritis.

People with bleeds can self-administer clotting factor, but if we are serious about joint health and improving the quality of life for people with haemophilia, researchers will need to look at other ways of treating besides administering factor. Some of these new directions include; better imaging, using novel biomarkers that might be able to reveal even minor joint bleeds, and by applying knowledge from other types of arthritis.

One problem is that there is no definitive way for physicians to distinguish between normal arthritic joint pain and that caused by a bleed so it will take research in all of these areas to work out new ways to diagnose and treat haemophilic joint disease and understand its causes.

Imaging technologies like magnetic resonance imaging (MRI), can help doctors identify bleeds but they are looking at quicker tools like ultrasound. There is a musculoskeletal ultrasound (MSKUS) system that can distinguish between bleeding and inflammation during painful episodes. MSKUS can check the crevices of joints for inflammation or bleeding, and the ultrasound can provide greater detail on what is happening where bleeding has caused both synovitis and inflammatory changes to soft tissue.

The cuase of haemophilia joint pain is still not clear. One hypothesis is that blood is a poor activator of a key protein called thrombin activatable fibrinolysis inhibitor (TAFI), which controls clot stability and reduces inflammation. Doctors in the USA are studying how treating patients with extra TAFI might help to relieve haemophilia joint problems. Researchers are also trying to better understand the contribution of clot breakdown in bleeding, and to investigate whether TAFI can be genetically modified to make it more potent and diminish bleeding complications.

This could lead to treatments beyond the standard infusions of clotting factor. If it is established that poor TAFI activation in haemophilia contributes to joint disease and inflammation, researchers could develop engineered 'high potency' TAFI that persist for longer in the body. The researchers hope that such agents could eventually mitigate or even prevent haemophilic joint disease.

Another idea being researched is a test that determines which people with haemophilia have the highest risk of developing joint disease.

Scientists have found biomarkers in the blood of haemophilic mice that could signal very minor bleeds before damage occurs in the joint. They can use this to create animal models for haemophilic joint degradation and this in-turn has lead to a scoring system to evaluate how well drugs stopped bleeding in the joints, which could be used to rank the effectiveness of new drugs.

These biomarkers could be used to predict joint deterioration or to guide scientists' search for new drugs to treat haemophilic joint disease, and could point to the fundamental mechanisms underlying the illness.

Prophylaxis with clotting factor gives good control of bleedsbut previous studies have shown that using extra doses of clotting agent to control bleeds that happen in between infusions (breakthrough bleeding) is only 40% effective, so it is not necessarily a good way to treat all patients with haemophilia. Just as you wouldn't treat an asthmatic with just an inhailer, patients with haemophilia could potentially be treated with drugs that reduce inflammation as well as being given clotting factor.

Another potential therapy is the use of special radioisotopes to attack the inflamed joint lining. Doctors have identified joint microbleeds in patients as young as two years old and if you can prevent that, 10 or 20 years down the road, they will be better off. One US company has developed a tin radioisotope technology that blasts out inflamed joint tissue. The company is completing animal testing for the tin-isotope project and are optimizing the technology for use in people.

This article is edited for size by the H Factor editor from an article by Katharine Gammon that appeared in Scientific American: *http://www.scientificamerican.com/article/haemophilia-s-crippling-joint-pain-is-a-target-for-scientists/*

2015 Annual general meeting

The 2015 AGM took place at the Toowong Library on November 17th. This was an opportunity for the foundation to present its activities from the preceding year to its members, to elect new office holders for the year to come, and for all the people that contribute to the foundation to touch base and also input into the direction for HFQ into the future. This year we were pleased to invite Maureen Spilsbuy who had just announced her retirement, to address the meeting.

The meeting was opened by Dave Stephenson who welcomed everyone. As the official parts of the AGM require a quorum, the meeting was officially postponed for half an hour as per the constitution and Maureen used this time to address the meeting.

This was a really interesting presentation full of enlightening insights and information from across her career as a haemophilia social worker. After her talk Maureen was awarded a life membership in recognition of her role over nearly 20 years of distinguished service. We are very sorry to lose Maureen and wish her well as she slows down a bit and focuses on other things important to her.

The AGM ratified the office holders and the managers report, and elected the new governing board. The reports are presented in the Annual Report which can be downloaded from the HFA website.

Craig Bardsley' regretfully advised the meeting that he was stepping down from the board; and because there were no challenges to the remaining office holders and board members, all were returned unopposed. The office holders for the 2015/16 year are:
President – Dave Stephenson
Vice President – Adam Lish
Treasurer – Peter David
Secretary – Leanne Stephenson
Board members – John Rowell; Erl Roberts; Robert Weatherall and Sara Hartley

All in all, this was a successful meeting and those that attended were rewarded with good company and useful updates. We look forward to welcoming everyone to the next AGM in 2016.

AGM President s Message

Members of the board continue to dedicate their time to lead HFQ in delivering services to those with coagulation disorders in Queensland and I thank them for their support during the year. The HFQ board has representation from all community demographics as well as professional experience in legal, medical and finance. A makeup which many boards would envy.

HFQ's priority is its members – people & families with coagulation disorders. HFQ is here for you in Representation, Education, Health Promotion and Support. I value your contribution through surveys and attendance at our activities – together I believe we all make a difference to our collective lives and health outcomes. The Queensland Government also recognises HFQ's outcomes and continues to provide funding for the current three year term and we acknowledge and thank them.

We are all aware Treatment products for coagulation disorders are extremely expensive (in the order of $220million nationally & Queensland government pays part of this). Engaging the community in their own health outcomes is a key part of maintaining not only health but value for money – it's the right formula.

I believe partnerships in health outcomes are the most effective way to achieve common goals. HFQ does this with a more formal partnership with the 'Queensland Haemophilia Treatment Centre' , HFA and other organisations where commonalities exists – this provides a wider focus on the broader issues affecting all those with coagulation disorders as well as targeting specific community and individual needs with Qld Health principles in mind.

HFQ services are driven by you, the community. This year we ran a successful community survey, the results feeding back into targeted services – refer attached activities report.

So as you can see, we had a busy and productive year and all with one paid staff member. I receive comments from others in the community sector who are very impressed with what we do with just one employee. Graham our manager, with support of the board, and working closely with QHC & HFA is the face of HFQ. His empathy, passion and intellect have driven better outcomes for individuals and community – well done Graham, the community thanks you!

Lastly I would like to thank you, the community for your support …. working together for common outcomes across Queensland. I look forward to an even better year ahead.

Bruise Protection

Avoiding trips to the emergency room with your toddler is an unspoken goal for every parent, but how do you protect your child from the potential for injury that comes with the territory when your child is learning to walk?

Develop a Philosophy

As the parent of a child with a bleeding disorder, one must consider the value of preventing injuries versus allowing exploration. Additionally, some parents weigh up the pros and cons of making young children wear protective clothing or a helmet, which may look awkward but provides a measure of protection.

It can be a "very tricky line" for parents to toe when children's natural tendencies are to get into trouble when they're newly mobile. Child experts suggest that on balance, it's much healthier for them psychologically to be able to interact with their environment the way all toddlers need for their growth. They're hard-wired to get into things they shouldn't and run before they're completely balanced, so they are going to fall down and potentially get bruised.

Anything a parent can do to protect a child with a bleeding disorder from typical bumps and lumps is important. But at the same time, try not to hamper the child's growth by being so protective that the child ends up fearful, frustrated or angry. Protective gear enables the child to explore his environment and play and have fun, that outweighs what may be perceived as stigmatising the child with a helmet or making him look funny with padded clothing. Some parents say it's better to risk your child be stared at occasionally than to have to spend a minute in the Emergency Department!

The severity of your child's haemophilia and a discussion with the QHC team are important when deciding about whether protective equipment is necessary.

Avoid Common Mistakes

Most toddlers who get hurt and require a trip to the ED have fallen off a bed or have run into a coffee table. The head is the part that you should be most concerned about, because it's bigger and heavier and that's generally what children hit when they fall. That's not to say

that toddlers cannot have joint bleeds, as well, so you need to be aware of elbows, ankles, knees and legs in little kids. Some specialists say that you should watch your child's gait for signs of discomfort if you suspect a joint bleed. And always watch for symptoms of a head bleed, such as vomiting, irritability, drowsiness, headache and confusion.

As parents, we can't protect our child 100% of the time, but some common-sense precautions can be used to minimise the chance of potentially dangerous accidents and head-on collisions. Be vigilant when arranging household furniture and consider padding sharp table edges, getting all floors carpeted or taping down rugs on hardwood or tile floors can help.

Helmets can be used by toddlers as they venture out into the world on two feet. Small children seem to like the helmets even if they cannot wear them all of their waking hours.

Options for Protective Gear

Parents of children with haemophilia and other bleeding disorders can be very creative in the face of necessity. If you hate the thought of using an "institutional helmet" around the time your child begins to crawl, think about solution that work for your child. Can you make him one out of thick cotton batting.

One parent did just this and then perfected her creation with high-performance -medical-grade foam and her the helmets are now sold online. (http://comfycaps.net). Your ideas may be just as good!

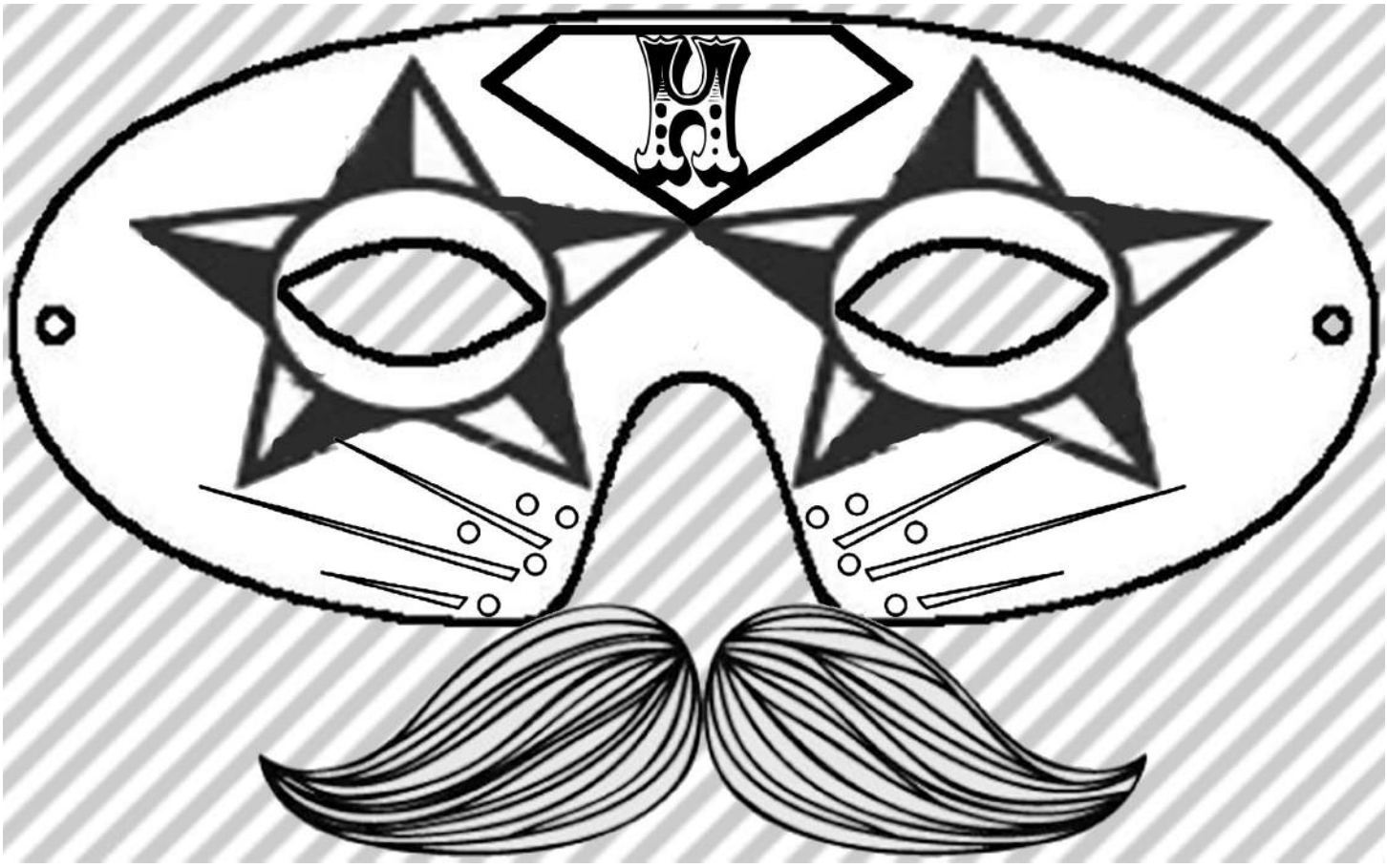
Think about where you need childproofing in your home. The crib can be a source of injuries, so you could pad it or buy sheets with inflatable sides.

Consider putting the thick blue interlocking foam play mats down on hardwood floors. They're great shock absorbers and easy to clean. You could also add gardening kneepads to the outside of pants or sew padding into their trousers.

The process of protecting your child from harm becomes easier with experience. It's better to be over protective at the beginning and discuss your options with the team at LCCH so you can be comfortable with your protective measures and know you've covered the danger areas for your child than be too cavalier and end up with an unwanted bleed.

Edited for size by the H Factor editor from an article by Kristina RAnderson that first appeared in Hemaware in 2007

http://www.hemaware.org/story/bruise-protection



= Fun Pages =

Don't sit around twiddling your thumbs these holidays! Pick up some pens, crayons, textas or whatever takes your fancy, and scissors if you've got your scissors licence, and read on!

I bet your mum, dad, relative or postman has called you their little superhero before! Well why not get one step closer by cutting out and colouring your own super hero mask?? Don't like ours? No problemo! Just put a piece of paper behind this one and cut a blank template ready for your own super hero colouring magic powers to be put in action!

JOKES

**Not guaranteed to make anyone laugh..*

*Getting sick of boring and extremely, very, not-funny cracker jokes? US TOO! Let us solve your holiday joke woes with 10 of the best (aka WORST!) cracker jokes you can whip out at your next party**

1. What do you get if you cross a bell with a skunk?
Jingle Smells!

2. What did the beaver say to the tree?
Nice gnawing you!

3. What athlete is warmest in winter?
A long jumper!

4. What song do you sing at a snowman's birthday party?
Freeze a jolly good fellow!

5. What is the best present in the world?
A broken drum – you just can't beat it!

6. Why couldn't the skeleton go to the party?
Because he had no body to go with!

7. Why don't ducks tell jokes when they're flying?
Because they'd quack up

8. What did the snowman say to the aggressive carrot?
Get out of my face.

9. What do you get if you cross a pig and a centipede?
Bacon and legs

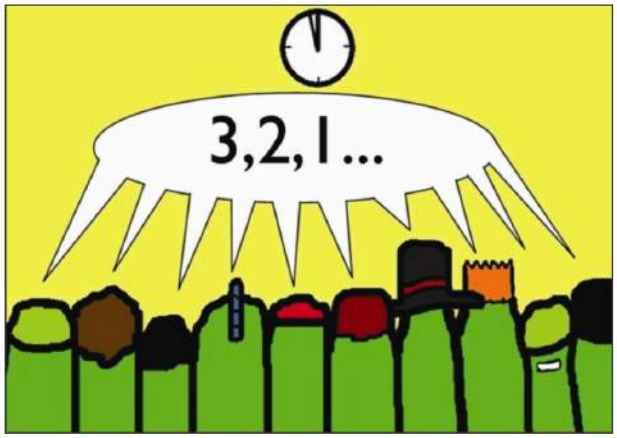
10. What do you call a line of men waiting for a haircut?
A barberqueue

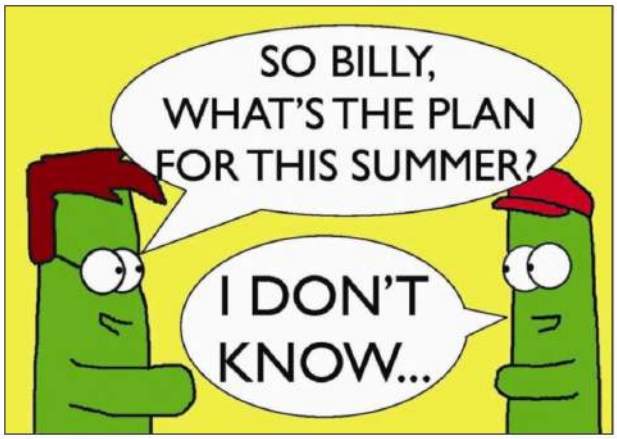
The Slobs

by Conor Birkett

First published by the Irish Haemophilia Society.

https://www.haemophilia.ie/content.php?id=7&article_id=698&level3_id=738





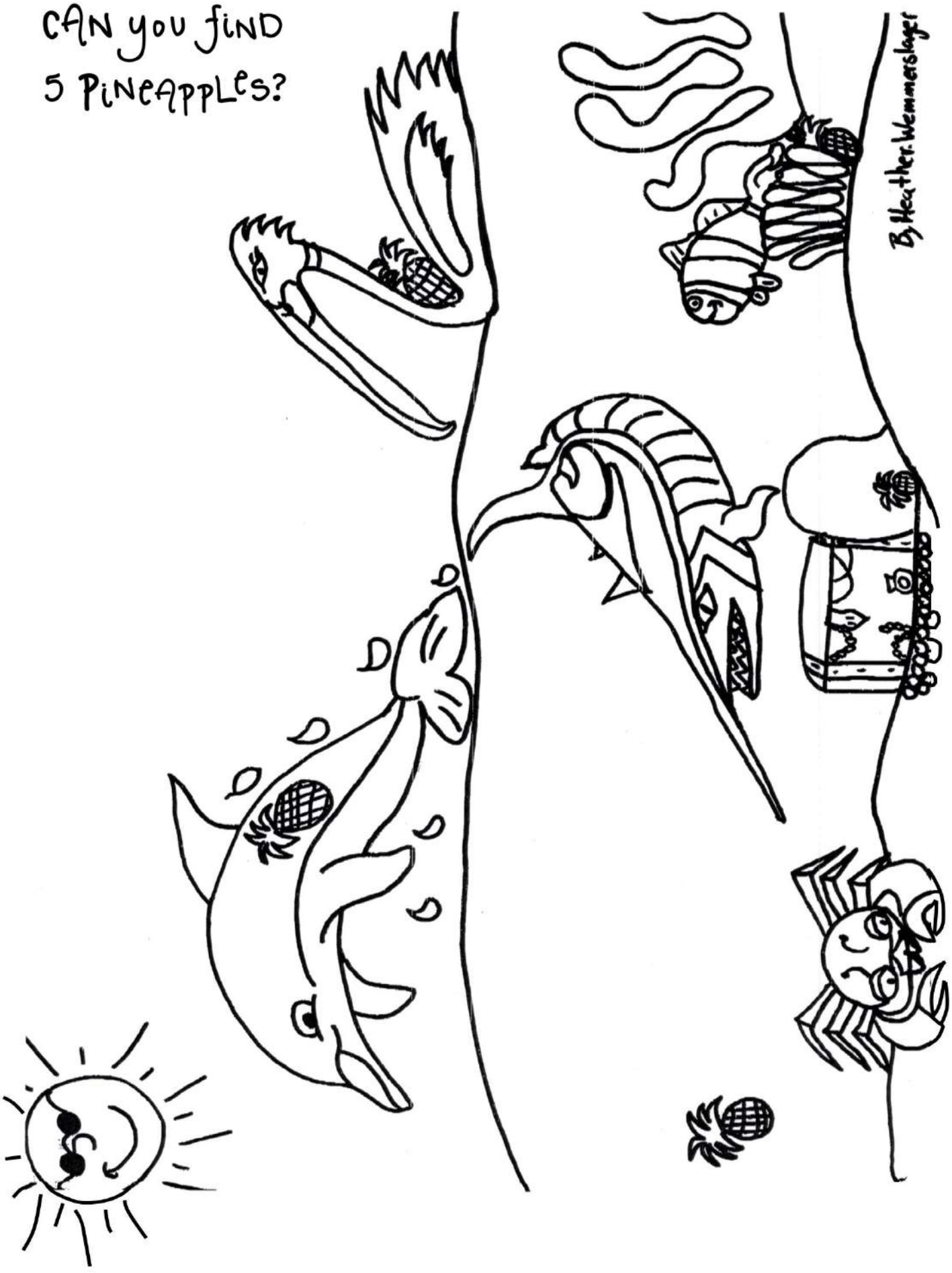
Did you know??

Humans aren't the only species on the planet who can be affected by haemophilia! Here are some (but not all) other species who have been studied for inherited as well as spontaneous instances of: haemophilia A: dogs, rats, sheep, cats; haemophilia B: dogs and cats; and Von Willebrands Disorder: pigs and dogs and cats. You may have heard about scientists using targeted gene disruption in mice to create haemophilia A, B, or VWD models. This is so they can study and better understand bleeding disorders and work towards a cure!

Finger-Walking Hero Puppet

Why not make your own super hero? Simply colour in and cut out (or hire a qualified professional scissors user) this super magical hero, put your pointer and middle fingers through the leg holes and up, up and away!

Life on the Reef—a colourful experience



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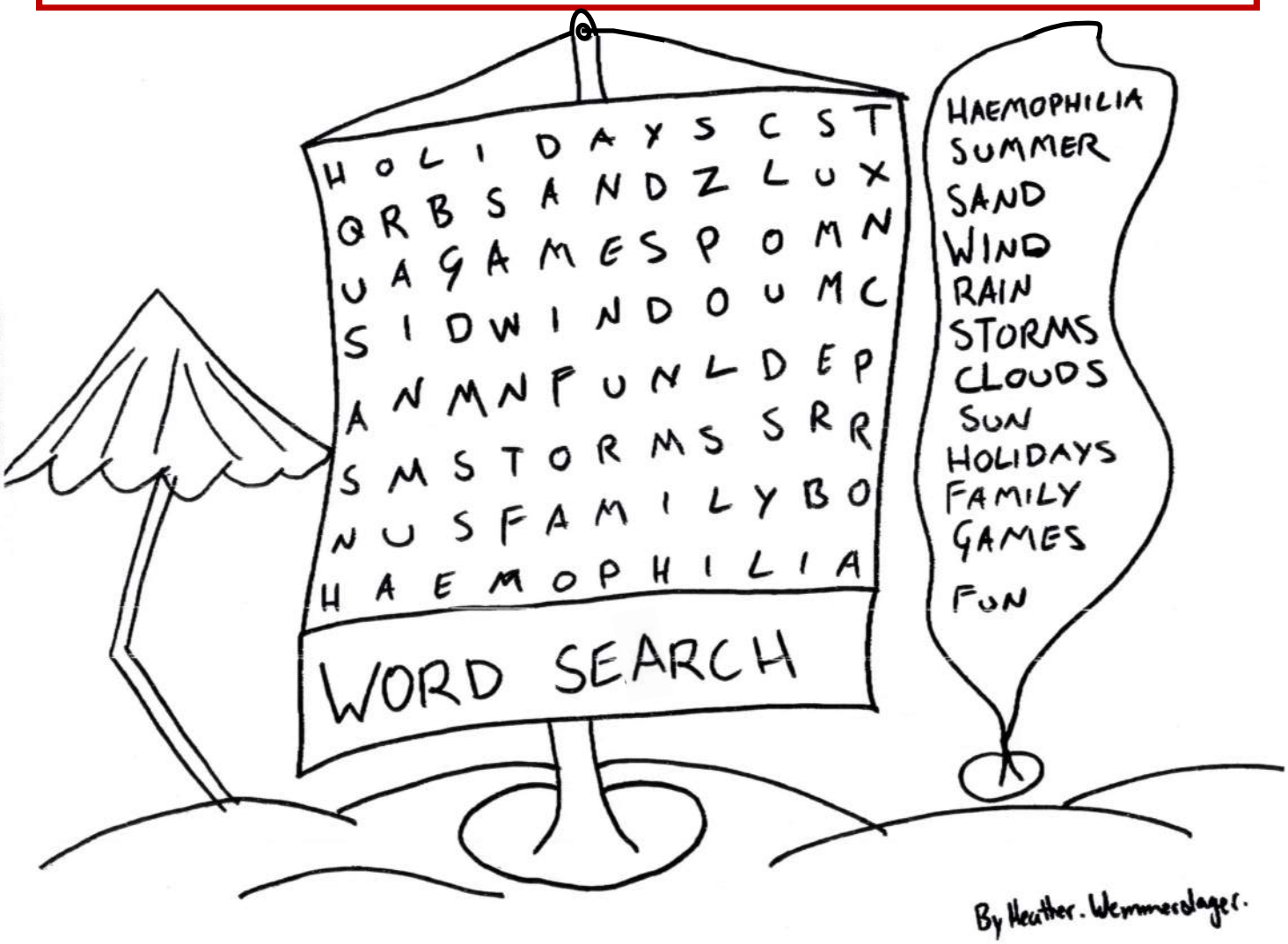


Tip for Toddlers

...continued from page 8

The following are some basic tips for parents of newborns and toddlers with haemophilia, von Willebrand disease or another bleeding disorder.

Tape or glue foam pads to the sharp edges of counters and coffee tables. Or better yet, remove coffee tables while your child is learning to walk. Foam padding on walkers will help protect elbows.

Use baby gates to block stairs - a good way to prevent falls.

Use netted crib covers to prevent falls out of bed.

Place non-skid strips on the floor of the shower or bathtub. Help your child in and out of the tub until the child is old enough to manage without falling.

Sew padding into the knees and seat of your toddler's pants and overalls to reduce bruising.

Make sure your toddler wears shoes to protect his feet. High-top sneakers provide good ankle support.

Consider getting your child a Big Wheel tricycle. They are generally more stable and closer to the ground than regular tricycles.

Buy athletic elbow pads and kneepads to help protect against joint bleeds caused by falls.

Make sure your child wears a helmet when skating, bicycling, etc.

Discourage physical activity that involves rough body contact, such as football, hockey and wrestling.

Enrol your child in the Medic-Alert system (HFQ offer member discounts to purchase these).

Talk with other parents about safety measures they use.

Getting Better Grades

If you want your child to bring home better grades, stop yelling and try this.

Right now, parents across Queensland are busy reviewing their children's school reports and thinking about their results. But what is the right way to respond to your child's report card – especially if your child isn't doing as well as you might like?

A recent US study asked parents of nearly 500 children how they would respond if their 11- to 13-year-old child brought home a report card with lower-than-expected grades or progress; and then investigated whether the parents' "punitive" or "proactive" responses predicted better or worse school results five years later.

The study found that children whose parents said they would respond by lecturing, punishing or restricting their child's social activities actually had lower levels of literacy and maths achievement by the end of high school.

The main reason that "punitive parenting" strategies like those are unlikely to work is that they do not directly address the underlying problems that lead to the poor result. It may also inadvertently deny your children the opportunity to learn the very skills and knowledge they require to improve their grades. Even worse, punitive strategies may increase children's sense of frustration and aversion to school work.

So what are some proven solutions?

On the positive side, the study shows that children growing up in a cognitively stimulating home environment – characterised by things like access to books, musical instruments, and trips to the museum – are likely to show higher levels of achievement in reading and maths in high school.

Other evidence also points to the value of creating a less punitive and more nurturing environment, though still with limits and boundaries for their children. Such an environment not only stands to enhance your child's academic achievements, but many other aspects of their development too.

Some research has shown the importance of giving and seeking specific feedback from a teacher, on what good performance is and how they can act to close that gap. Teachers are a great source of information so that parents can understand the reasons behind their child's poor performance.

Teachers are not only there to help, but are an important ally in helping improve your child's school performance. Engage in co-operative and constructive collaboration with your child's school that is built on mutual respect and understanding.

It is important to note that there are plenty of other factors that can predict academic success: genes, parents' level of education, the age of parents when a child is born, school infrastructure and teacher performance.

Some of these factors can't be changed, but many can.

The challenge for parents is to tune in to those things that can be changed and act on them accordingly.

Three tips to remember at report card time

When unexpected or poor results come in, research shows that reacting with frustration, anger, lecturing or punishment isn't the best way to get better results.

Consistent and responsive parenting will do more good than a punitive approach.
Give and seek specific feedback on your child's progress – especially the reasons behind any unexpected results.

Written by John Pickering. Head, Triple P Innovation Precinct, The University of Queensland. and Jinny Hong. Research Officer, Triple P Innovation Precinct, The University of Queensland

First published 29 October 2015 https://theconversation.com/if-you-want-your-child-to-bring-home-better-grades-stop-yelling-and-try-this-48975

THE CONVERSATION

WFH Assessment on Inhibitor Studies

The World Federation of Hemophilia updated their information on the results of several articles in peer-reviewed scientific journals suggesting elevated inhibitor rates for Specific Recombinant FVIII Replacement Concentrates in previously untreated patients (PUPS) with haemophilia A (when compared to other recombinant Factor VIII products). This is the information Dave refers to in his Presidents Message (cover page) and it is reprinted here for your information. ..

Third publication suggests recombinant FVIII product associated with higher risk of inhibitor development in newly diagnosed, previously untreated patients with severe haemophilia A

On 3 October 2014, the World Federation of Hemophilia (WFH) issued a communiqué regarding a study published by a group in France that demonstrated a higher than expected incidence of inhibitor development in previously untreated patients (PUPs) with severe haemophilia A treated with Kogenate FS/Bayer/Helixate NexGen compared to other recombinant factor VIII (rFVIII) products. Since then, a study published by a group from the UK has reported similar findings.

These results follow on from the unexpected results in the RODIN study, published in January 2013. That study was reviewed by regulators and in December 2013 the European Medicines Agency's (EMA) Committee on Human Medicinal Products (CHMP) endorsed recommendations which concluded that the benefits of Kogenate FS/Bayer/Helixate NexGen continue to outweigh their risks in PUPs with haemophilia A. The EMA stated that the product information for this product should be amended to reflect the results of the RODIN study and clarify that there is no different risk between products.

Inhibitor development is caused by many risk factors, which makes it difficult to draw conclusions in a small patient population. At the moment, no firm conclusion can be made. The WFH has 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 have confirmed that they will be re-examining the data. In January 2015 the EMA confirmed it will work with the investigators of all of the relevant studies regarding

Kogenate FS/Bayer/Helixate NexGen and inhibitors development in PUPs to make a recommendation for safe and effective use. This review is expected to take several months. It is the view of the WFH that all of the available data should be pooled in order to give a clearer answer about the relative risk for individual products.

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.

The WFH will closely monitor this situation and will communicate again when further relevant information is available.

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

National Hemophilia Foundation Medical Advisory #417: MASAC Assessment on Inhibitor Studies can be found at: *http://www.kintera.org/cms.asp?id=6728905&campaign_id=171801&enString=ddRyOrQjJjIEKQPoF4KEJPOrFdKtGvPaOORCNZMtFcKJIXMpF8LSG*

Travel Tips

Some of you will be travelling to be with family and friends these holidays and there are a few things people with bleeding disorders can do to help ensure they're prepared before hitting the road. Here are some tips we posted these to our Facebook page...

DISCUSS TRIP PLANS WITH THE QHC TEAM.
Travel can be stressful and cause changes in you or your kids activity levels. Adjustments may be necessary. For example, if visiting the beach or aquatic centre you may need to increase your therapy or change the times you take it so you've got good factor levels when your most active.

FIND OUT WHERE THE NEAREST HTC AND/OR HEMATOLOGIST IS (if travelling interstate). Or at least be familiar with the local Emergency Department because emergencies happen especially when on holiday! Trying to locate doctors and hospital ED during the emergency itself is never a good idea. Know where you're going before you need to go. Ask us if you're not sure where to go.

ALWAYS TRAVEL WITH FACTOR!
Even mild bleeders could prepare with an emergency dose. More severe bleeders should anticipate their factor needs and include an emergency dose or two. If you travel, consider counting out your prophylaxis doses and include an extra dose for each week that you're going to be away.

PLAN FOR PROPER BIOHAZARD DISPOSAL.
Seriously, what will you do with those used butterflies and other infusion material? Sharps containers come in several different sizes. Get a small one if you need it and included it with your supplies for travel. Then it won't weigh you down.

CANCELL YOUR FACTOR DELIVERY.
Check your diary for when your next scheduled Factor delivery will occur. If it's due to be delivered while you're away ring the delivery team and get it rescheduled.

PREPARE FOR THE AIR!
If you're flying (especially overseas), there are just a few extra things to remember:

GET A LETTER FROM THE CLINIC OR YOUR GP that explains your bleeding disorder and medicine requirements. Make sure the letter is official (on letterhead with phone numbers) so airport security don't hold you up asking about the butterflies, needles or medication!

Finally; people flying with a bleeding disorder should **NEVER LEAVE FACTOR IN CHECKED BAGGSGE!** Keep it with you on the plane. Lost bags can happen and this would immediately ruin your grand travel plans. What good is a trip to your grandma's in Melbourne, without the medicine you need? You can survive without a change of clothes, but not without factor.

So there you go! Have a great holiday. If you're travelling, don't be a turkey and leave unprepared. Factor in factor for your travel plans and have a great journey

Physiotherapy

Physiotherapy has proven benefits for people with bleeding conditions, that's why they are a key component of the comprehensive care team at your haemophilia treatment centre. From personal experience, peer reviewed articles and talking to others who have been involved with physiotherapy treatment I would recommend anyone with a bleeding condition to have a review at least once a year. I was recently attending the physiotherapy department at the Royal Brisbane Hospital where the use of the 'Rehab Ergometer trainer' that HFQ donated was in use.

This is a device that is used for upper and lower body training and used to achieve improved outcomes for people by matching / adjusting exorcise intensity. It is important to note that a good understanding of bleeding conditions is essential – if you are treated by a physiotherapist without knowledge and experience of bleeding conditions, in my view you are taking a risk. So, remember, just like your annual GP check-up, make an appointment with your haemophilia treatment centre.

HFQ – achieving outcomes for those with bleeding disorders.

Conference Briefs

From the 1 to 3 October HFQ members attended the Australia & New Zealand Conference on haemophilia and related bleeding disorders on the Gold Coast.

This is always a stimulating and educational event, and this year there were some great sessions including;

- ⇒ Comprehensive Care;
- ⇒ Tools & Strategies to support your child;
- ⇒ Genetic Counselling;
- ⇒ Hepatitis C & HIV;
- ⇒ Pain in Haemophilia;
- ⇒ Inhibitors;
- ⇒ Mild Haemophilia;
- ⇒ von Willebrand Disorder;
- ⇒ Women Bleeders; and
- ⇒ Making informed family planning decisions.

In the Comprehensive Care session the presenters looked at what comprehensive care for bleeding disorders could look like. They argued that treatment must be effective, appropriate, sustainable and affordable and pointed out that Australia, is yet to establish an audit process for to see if we are meeting these standards of care.

There was also a very interactive youth session ranging from taking responsibility for your own life, treatment, and contact with the HTC; to informing an employer, about your bleeding disorder. It became clear that it is important to know your legal rights as people had good and bad experiences as a result of informing employers. .

A/Prof Strasser presented on Medical Issues for PWBD and Hepatitis C treatment. She stressed the importance of Lifestyle Management and; the need to reduce risk factors (cease smoking & alcohol, reduce weight, and increase exercise). Her message was to treat now. Although there can be medical barriers to treatment, patient barriers such as lack of awareness of a potential cure, lack of confidence in therapy, feeling well at present, fear of side effects, and competing priorities were all significant contributors to delaying treatment.

Profs Mosely & Arnold presented an amazing session on Improving Outlooks for Pain in Haemophilia. They explained that pain is an output of the brain, not an input. It is the warning the brain gives when it determines that an injury is sufficiently bad, or causing enough potential danger, to warrant one.

Chronic pain involves significant neurological changes and once these changes occur, recovery is much more difficult. Persistent pain should not be ignored. Deal with it sooner, rather than later. The brain cells that produce pain get better and better at producing pain. They become more and more sensitive.

Pain management must be holistic. You should learn to think about pain differently, and be aware of the influence that we have over our brains.

We hope to post more on what can help with pain in the next issue — Ed

The session on inhibitors discussed the consequences of inhibitors and how the demands of treatment increased the burden on patients and could lead to reduced quality of life, financial stress, and strained relationships. Improved management of patients with haemophilia complicated by an inhibitor is a challenge for the health care community as the main support system for these individuals and families.

Dr Iorio argued that all, studies seemed to end in the same outcome – 30% development of inhibitors. He said that reactions at this rate are not considered "rare" adverse events and that it's better to ask why 2/3rds of patients DO NOT develop inhibitors if we want to further understand this issue.

Dr Wilson said that genetic testing for a predisposition to inhibitor development is a good idea, and that her study found that the Australian switching of products was not associated with inhibitor development.

The final speaker in this session was Dr John Rowell who said that 50% of inhibitors are transient and will decrease or disappear. However he also pointed out that inhibitors are the most significant cause of death for those with factor 8 deficiency.

There were also some great sessions on mental health. Dr Ira Van der Steenstraten presented on mindfulness. Training your attention and doing selected exercises can reduce stress, anxiety, and depression, boost the immune system, and strengthen your ability to focus your attention where you want it to be. It has even been shown to change the architecture of your brain

Evidence has shown that meditation no longer just for "hippies" and we are offering a workshop on this next year (see page 6 for information).

Other mental health issues covered were bullying and resilience. Children can be bullied irrespective of having a bleeding disorder, but adults need to take it seriously if a child is telling them about, or showing signs of, being bullied.

Increasing resilience in children is also important as it is a key part of dealing with bullying and is the process of adapting well in the face of adversity, trauma, tragedy, or significant sources of stress — such as family and relationship problems or serious health problems.

For a child with a bleeding disorder resilience is vitally important. They have to learn to contend with medical procedures, being poked and prodded, missing out on being in school activities and …. just being different to others .

First recombinant von Willebrand factor

FDA approves first recombinant von Willebrand factor to treat bleeding episodes

The U.S. Food and Drug Administration has approved its first recombinant von Willebrand factor (Vonvendi), for use in adults 18 years of age and older who have von Willebrand disease (VWD). Vonvendi is approved for on-demand (as needed) treatment in the US to control bleeding episodes in adults diagnosed with VWD.

VWD is the most common of the inherited bleeding disorders. It affects approximately 1 percent of the U.S. population and a similar amount of people here in Australia. Men and women are equally affected by VWD. Just like Haemophilia, patients with VWD can develop severe bleeding from the nose, gums, and intestines, as well as into muscles and joints. Women with VWD may have heavy menstrual periods lasting longer than average and can experience excessive bleeding after childbirth.

Vonvendi underwent two clinical trials (69 adults with VWD) which showed it to be safe and effective for the on-demand treatment and control of bleeding episodes from a variety of different sites in the body. No safety concerns were identified in the trials.

Karen Midthun, M.D., the director of the FDA's Center for Biologics Evaluation and Research said that the approval of Vonvendi provides an additional therapeutic option for the treatment of bleeding episodes in patients with von Willebrand disease. We don't know when it will become available here in Australia but members with VWB should discuss with the Queensland Haemophilia Centre if they have concerns about blood loss and ways they can reduce it.

The FDA granted the recombinant von Willebrand factor an orphan designation which provides incentives for the manufacturer to continue to develop treatments for rare diseases
Such as bleeding disorders

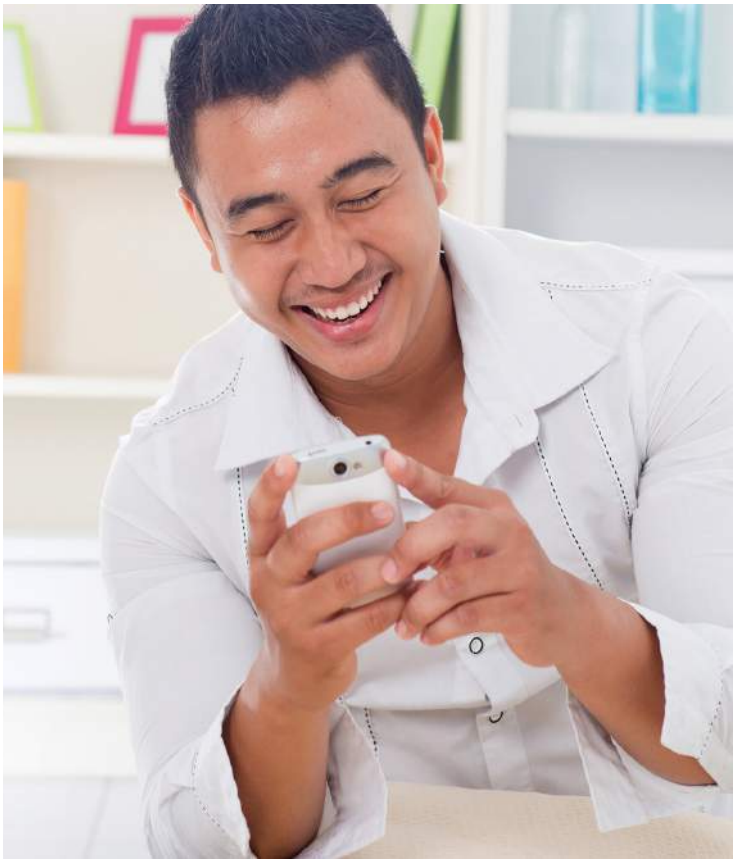
Edited for size from an FDA News Release : http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm476065.htm

My ABDR Beta Testing

The MyABDR development team at the national Blood Authority is looking for volunteer MyABDR app users to test some new enhancements in mid-January 2016. This will involve downloading a 'Beta' version of the app and using it like their usual app, but testing specific aspects and sending through a feedback email each time they test.

We have reached a critical point with MyABDR development. The NBA is investing in another round of development and it is essential that we have community input to make sure these enhancements work for our members. If you are using the MyABDR app and are willing to help please contact Suzanne at HFA to be included (or for more information) on 03-9885 7800 or email: socallaghan@haemophilia.org.au

If you're not recording your use of factor or bleed incidents, now is a good time to consider it again. Make it your new years resolution because this is a tool designed for our members to make treatment conversations easier and that way the clinic team can match and meet your needs more easily



Tolerance to Clotting factors from Lettuce

Drugs from lettuce may promote tolerance to clotting factors

Freeze-dried lettuce leaves are a surprising new source for shelf-stable drugs, report researchers.

Biopharmaceuticals, or drugs that are based on whole proteins, are expensive to make and require refrigeration to store. Insulin, for example, is unaffordable and inaccessible to most of the global population.

At the University of Pennsylvania School of Dental Medicine, researchers have been working to overcome these obstacles by using a plant-based system to make shelf-stable drugs.

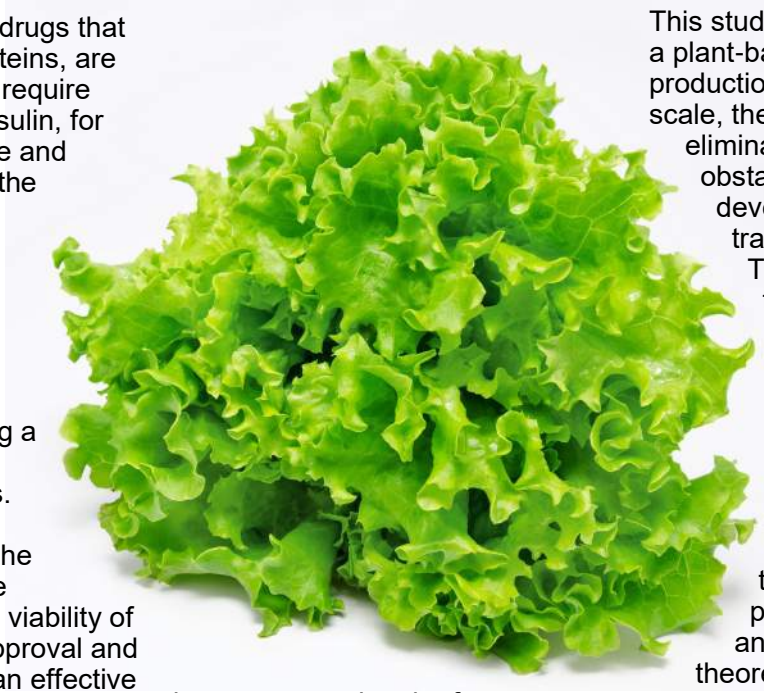
In a study published in the journal Biomaterials, the researchers confirm the viability of their method for FDA approval and human use, producing an effective drug that promotes tolerance to clotting factors, which could be taken by haemophilia patients, using freeze-dried lettuce leaves.

This is the first time a group has shown the commercial viability of producing a low-cost drug made from whole plants.

The study builds on previous work by senior author Henry Daniell (a professor in the biochemistry department) and his group demonstrating an ability to use genetically modified plants to introduce a protein into the body that would teach the immune system to tolerate clotting factors used to treat haemophilia.

The study used a lettuce platform to "grow" the drug. After identifying a compatible vector, they used a similar protocol to their previous work, bombarding lettuce leaves with a fusion of the therapeutic protein, coagulation factor IX, or FIX, with cholera toxin B subunit, which allows the protein to reach the immune system.

They then evaluated the resulting plants for those that took it up and then grew those plants to maturity. The researchers were able to harvest a new batch of pharmaceutical-containing lettuce every four to six weeks.



The next step was to ensure that the drug would be shelf stable. To do that, they freeze dried the plant material, ground it, and analysed the resulting fine powder for expression levels of the fusion protein to determine the appropriate dose and to evaluate its efficacy.

Similar to their previous experiments, Herzog's lab fed haemophilia B mice with a suspension of plant cell containing clotting factor IX twice a week for eight weeks and then gave them the same clotting factor that human haemophilia patients take to encourage blood clotting. As before, their product was a success: mice given the drug had greatly suppressed inhibitor formation compared to untreated animals, even when various doses of the drug were tested.

"One of the key findings of our study was that we found our drug was efficacious across at least a 10-fold dose range," Daniell says.

Stable for two years

This study confirms the viability of a plant-based biopharmaceutical production on a commercial scale, the researchers have eliminated several expensive obstacles that hamper the development of affordable traditional protein drugs. The method requires no fermenter, no purification to ensure sterility, and no cold chain to keep the drug refrigerated.

In addition, the researchers found that their capsules remained potent and effective for two years, ensuring the product is shelf-stable and patients could theoretically take the drug from home.

"Current treatments for inhibitor formation in haemophiliacs cost almost a million dollars and are not affordable for a significant segment of the patient population," he says, "but the new drug is dramatically cheaper and may offer even a better solution for treating haemophilia patients. Most important, developing a low-cost platform for protein drug delivery will make these drugs affordable for a large majority of the global population."

Edited for size by the H Factor editor from an article published at:http://www.futurity.org/lettuce-drugs-hemophilia-1028162-2/
Posted by Katherine Unger Baillie-Penn on October 19, 2015

More Genetic News

Rabbit milk may offer a haemophilia cure

LFB USA Inc. Is a biotechnology company that has already proved that goats can be used as a barnyard drug manufacturer is adding the rabbit to its production line. It has genetically altered the rabbits to produce milk containing Factor VII.

The expansion has been years in the making, and is part of LFB's preparations for a product that, if approved, would compete against current haemophilia treatments. LFB's French parent company, LFB SA, is already in the final stages of testing its Factor VII in humans, using material made by rabbits in France.

LFB's focus has always been on harnessing animals to make materials for human medical treatments and the president of the US operations says that are getting themselves positioned for launch and commercialisation of the product.

The company's goats produce the human clot-busting protein antithrombin III in their milk which is used to prevent clotting problems before surgery or childbirth in patients who lack antithrombin.

But when LFB decided to pursue a Factor VII product, however, it concluded that goats wouldn't work. The pattern of carbohydrates that goats deposit on proteins in their milk just wasn't right for Factor VII.

But rabbit milk did the job, rabbits present certain advantages as dairy animals. Females, or does, can begin reproducing at about five to six months old. The New Zealand white rabbits used by LFB also have big litters of about 10 kits.

Once they've given birth, does can produce milk for about three weeks.

Breeding rabbits is also relatively simple, and they are cheap to house and maintain.

At LFB's rabbit milking farm, the company houses 410 rabbits in a new 12,000-square-foot facility with about eight to 10 workers tending the animals.

The rabbit milking operation is very different from LFB's goat farm. Goats live in barns and play on rocks in outdoor corrals. They also line up for milking in a way that similar to dairy cows.
But rabbits are more particular than a lot of other species. LFB keeps its rabbits indoors to avoid exposure to disease and milking them is also unique.

Workers are clothed in sterile garb and milk the rabbits by putting them into a table top hammock and securing the animal with a flap that looks a bit like a coat a dog might wear for winter walks, before attaching mechanical pumping tubes to the doe's teats. Milking takes about six minutes. From there, does go to rolling carts with open containers, then travel back to cages in another room.

Groups like the U.S. Humane Society don't support the use of transgenic animals, and have expressed concern that animals might get milked and handled more than they should, and they might suffer stress or pain.

But LFB does not hide what it does, which is producing life-saving drugs in dairy animals and says animal health and welfare is paramount , so it does seek to work with animal welfare groups to alleviate any concerns.

Workers often stroke the rabbit's head and ears while it's being milked and the cages contain perches and balls , where the rabbits can climb and play.

LFB says the rabbits are the result of years of work. They are aiming to breed the rabbits, and have about 1,000 animals on site by 2017.

The company says its all in an effort to address inhibitor development in haemophilia patients and the company's rabbit-made Factor VII would compete against Novo Nordisk's NovoSeven as well as plasma-derived Feiba from Baxalta Inc.

Edited for size by the H Factor editor from an article published in Telegram.com by Lisa Eckelbecker
For the full article see: http://m.telegram.com/article/20150913/NEWS/150919640

Controls for HIV

December 1 was (and always is) World AIDS Day. We don't always like to talk about some of the things that haemophilia has brought into our community but it's a fact of life that some of our members received factor derived from blood products infected with HIV and other Blood Bourne Viruses (BBV's).

The blood supply is now far better protected than it was in the mid 1980's and the factor we get now is seldom made from human derived blood products. But we still have to deal with the consequences of these infections and it's regrettable that HIV and HCV can still be acquired by members of our community just as they can the general public, but it's exciting that science is finding solutions for these conditions too.

World AIDS Day is a time to raise awareness in the wider community about the issues surrounding HIV and AIDS. It is a day to demonstrate support for people living with HIV and to commemorate those who have died. It is also a time when we remember the members of the bleeding disorder community who were affected by HIV. The legacy of this tragic episode lives on in our community, especially for the people who lost partners, family members, children, patients, colleagues and friends.

Some people with bleeding disorders continue to live with the challenges of HIV; and we acknowledge the individuals who inspire us by their positive attitude, resilience and determination to build a better future.

In 2015 the World AIDS Day global campaign continues the theme of:
Getting to Zero

- Zero new HIV infections
- Zero discrimination
- Zero AIDS related deaths

What can you do?
HFQ sits on several bodies that look at these infections in Queensland and we input into research and support options on behalf of our members. You can also help;

- Be aware that HIV still exists in the community
- Take action to prevent transmission of HIV by promoting safe sex practices
- Support and understand people in your community living with or affected by HIV
- And uphold the right of people living with HIV to participate in the community free from stigma and discrimination.

If you want to know more about this please talk to Graham at the office on 0419 706 056.

Treatments for HIV are now available that can keep HIV under control and this keeps your immune system strong. Genetic research is also impacting on scientific understanding for this condition, like it does with bleeding disorders themselves. This means that there may be a complete cure one day soon.

An example of this is a recent article in AIDS Meds that reported a genetic research companies efforts to develop genetic therapies for HIV are having some early success, with two of three participants in one cohort maintaining control of the virus for an extended period during which they were not taking standard antiretroviral (ARV) treatment. The biotech company (Sangamo Biosciences) is conducting a Phase I/II study, of its genetic treatment, called SB-728-T. Updated results about what is known as Cohort 3 of the trial were presented at the recent Interscience Conference of Antimicrobial Agents and Chemotherapy (ICAAC).

Cohort 3 includes three HIV-positive participants who received a genetic treatment in which their own CD4 and CD8 immune cells were drawn out, genetically modified to resist HIV, and then infused back into their bodies. This treatment was different from what was given to other participants in the study because it included modified CD8 cells instead of just modified CD4s.

After undergoing the genetic treatment, the members of Cohort 3 all stopped taking ARVs. Two of them have maintained control of HIV for longer than 16 weeks.

The companies Vice President Dr Dale Ando said in a press release that the ability of subjects treated in Cohort 3 to suppress and sustain control of viral load, combined with durable increases in CD4 and CD8 cells, provide support for our hypothesis of an immunologic mechanism of action for their genetic treatment. "The prolonged positive effects observed in these Cohort 3 subjects have not been seen before with other treatments and have encouraged us to enrol and treat an additional five subjects with this regimen."

Written by the H factor editor. Info on genetic treatment SB-728-& is from an article at: http://www.aidsmeds.com/articles/Sangamo_gene_treatment_1667_27834.shtml

Health Updates

SIPPET Results Released

Some results of the SIPPET (Survey of Inhibitors in Plasma-Products Exposed Toddlers) study were published on 11 November as a conference abstract in advance of the American Society of Hematology (ASH) Annual Meeting this December. The abstract suggests that, in previously untreated patients, 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. The WFH will make further comments after the publication of the complete study in a peer-reviewed journal.

The abstract can be viewed at https://ash.confex.com/ash/2015/webprogram/Paper82866.html

Value Added Role of Physiotherapy

The New Zealand National Bleeding Disorder database was surveyed to determine the costs of using coagulation factor VIII by people with bleeding disorders treated by the centre prior to the appointment of a specialist haemophilia physiotherapist and after two years of a haemophilia physiotherapy service. They found the physiotherapist added value to the Haemophilia Care Team. KPI's were set to assess all patients within 24 hours of reporting a bleed to help to prevent treatment of arthritic pain with factor replacement products.

People with inhibitors, haemophilia B, symptomatic female carriers and people who moved between 2010 and 2013 were excluded from the analysis. No adjustments were made for changes in weight during this period. Between 2010 and 2013, total factor VIII product usage fell by 342,712 IU representing a 6.8% reduction. 100% of the patients who completed a patient satisfaction survey (70% response rate) were satisfied or very satisfied with the physiotherapy service and they agreed or strongly agreed that a designated physiotherapist service had improved the quality of their care.

Source: http://www1.wfh.org/docs/en/Events/MSK-2015/Abstracts_MSK2015.pdf

Gene Therapy Trial of Haemophilia A Treatment Enrols First Patient

BioMarin Pharma (a WFH Corporate Partner) has started a Phase 1/2 trial for an investigational gene therapy (BMN 270), for the treatment of hemophilia A. BMN 270 is designed to restore factor VIII plasma concentrations. For the first clinical trial they are looking to demonstrate that treatment with BMN 270 increases the expression of the factor VIII protein, necessary for blood clotting.

The Phase 1/2 study will evaluate the safety and efficacy of BMN 270 gene therapy in up to 12 patients with severe Haemophilia A. The company is looking to assess the safety of a single intravenous administration of a recombinant AAV, human-coagulation Factor VIII vector and to determine the change from baseline of Factor VIII expression level at 16 weeks after infusion. This is a dose escalation study with the goal of observing an increase in Factor VIII levels. Secondary endpoints include assessing the impact of BMN 270 on the frequency of Factor VIII replacement therapy, the number of bleeding episodes requiring treatment and any potential immune responses. Patients will be monitored for safety for five years.

For more information see: http://www.streetinsider.com/dr/news.php?id=10925239

A Gene Silencing Therapy for Haemophilia

Sanofi's Genzyme unit has opted into a new drug candidate called ALN-AT3 for haemophilia. It is an RNA interference (RNAi) drug that works via a process known as gene silencing, switching off the function of an endogenous protein called antithrombin (AT). The drug, is administered by subcutaneous injection once a month in patients with moderate-to-severe haemophilia and interim results showed that it was able to reduce the activity of AT by up to 86% which is an integral part of the clotting process, as well as a reduction in bleeding frequency and the effects lasted over two months after the last dose was administered.

On the strength of these results the company intends to advance ALN-AT3 directly to a phase III trial in mid-2016. If it proves effective in late-stage trials, ALN-AT3 could have a dramatic impact on haemophiliac patients who develop inhibitory antibodies against standard therapy with blood-clotting factors VIII and IX.

For more information see: http://www.pmlive.com/pharma_news/genzyme_opts_into_gene_silencing_therapy_for_haemophilia_832710?SQ_DESIGN_NAME=2&

Important Dates for HFQ Members

Summer Burgers by the Pool. Australia Day event (26 January). Chermside Waterpark from 10 till 3.

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

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

Go For It Grants

Have you ever wanted to be daring and take on a challenge?
Or do something that's a bit scary?
Or try something new?

Everyone experiences obstacles at some time, but you'll never know what you can overcome and achieve unless you Go for it!

The Haemophilia Foundation Australia's new **Go for it Grants program** aims to assist people living with bleeding disorders take the first step towards achieving their goals. The grants are open to people affected by haemophilia, von Willebrand disorder and other rare inherited bleeding disorders of all ages. That is; **anyone who has, or is affected by, a bleeding disorder**.

There are **2 x $5,000 Go for it Grants** on offer to provide winners with the financial support to help pursue their dreams.

What do you want to try? From study to advance your career, increasing your confidence and leadership skills with a public speaking course, or training to be the next sports star - the Go for it Grants can take you one step closer to realising your dream.

For a copy of the guidelines or to apply, download the application form from the HFA web site - www.haemophilia.org.au Or contact HFA on 1800 807 173, email hfaust@haemophilia.org.au .

Applications close 10 January 2016 so Act Now Don't wait – just Go for it!

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. You can be removed from the list at anytime.

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