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The H Word

Kia ora koutou,

As the year comes to a close, we finish on a high note, with a strong and dedicated HNZ team, national events in the pipeline, a solid financial position, a 'home' for our organisation, and emicizumab (Hemlibra) now available for all members with severe and moderate haemophilia A.

Our board of committed volunteers continue to devote time to advancing the organisation's mission. Thanks to Catriona and Karl (vice-chairs) Hemi (treasurer) Tineke (delegate), and Lauren (youth delegate) for your time on the board this past year. I would also like to welcome Connor McCone, who has joined the board as the new youth delegate. Thank you for offering your time for this important role. I would also like to acknowledge the crucial role Kiwifirst plays as our chief fundraiser. Thank you to all the staff at Kiwifirst who work tirelessly, and the public who loyally donate so it is possible for us to support our members. I would also like to acknowledge the support of our corporate partners and benefactors.

With the appointment of Vic Turner to HNZ, we once again have a full outreach team. Vic works alongside Lynne, Darian and Loren and builds connections both within respective regions and across the country. Phil and Leanne continue to keep the day-to-day running of the organisation on track.

With the dedication of the staff and Board we were able to reinvigorate our events programme. These events are central to our ability to connect and educate our community. We now have a fixed calendar of national events lined up until 2025. Coming up in March we have our teen and youth camp, followed by women's wellness weekend in July. Keep your eye out for invitations to these events.

I conclude my report with the most significant announcement for our members: As the year ends, all members with moderate and severe haemophilia A now have access to Hemlibra. Pharmac began funding this very effective treatment on October 01. This represents the most significant change and improvement to therapy for haemophilia in more than 25 years.

With this announcement in mind, I enter the new year thankful for the support of our staff, volunteers, and members, and looking forward to the development of our new home for HNZ.

On behalf of the team at HNZ I wish you and your whānau a restful festive season, and all the best for the New Year.

Deon York.

Board Chair and Acting CEO.

Now we're HNZ

On February 27 1958 the NZ Haemophilia Society was formally incorporated. This represented the culmination of several years of hard work by people affected by bleeding disorders and their whānau across NZ. On September 11 1979 NZHS became an approved charity. Then, on January 26 1999 our name was changed to the Haemophilia Foundation of NZ. Now we're Haemophilia NZ!

On Thursday 10 August, at a special general meeting, those in attendance voted unanimously to change our name and update our logo. This was another milestone in our history, and a fitting accompaniment to the purchase of our new home in Wellington.

We've been operating as the Haemophilia Foundation of New Zealand since the late nineties, and our branding has essentially stayed the same since then too. The Board, following consultion with a cross section of members, engaged Hive Creative to design a logo and associated assets that reflect the aspirations of our organisation. We are very please with the designs they came up with. You may already have noticed these images appearing around the place. Our Pānui enewsletter has been made over, as has Bloodline. We have also applied the new branding to our website, which you can see here: https://www.haemophilia.org.nz/

Haemophilia New Zealand

Hive Creative design consultant Manukorihi Winiata describes our new logo:

"The idea was to create a simple tohu that communicates a sense of growth, togetherness, embrace, care, and support

This is inspired by the double Helix and exploring how it connects to Whakapapa and whānau. Connections, Education and transformation were the key drivers.

The interweaving effect creates different dimensions representing diversity. It acknowledges that the Haemophilia community are people from all walks of life making connections and transforming together. It shows the overcoming of hardship.

The koru pattern can be associated with growth, wellbeing and strength. There are two koru that are designed back to back. This talks about the passing over of knowledge to allow growth and transformation, while being supported through that process.

The direction of growth indicates the past, present, and future. Acknowledging those in the past who did the ground work, to the present day, and also the future to come."

Over the coming weeks you'll see more and more of the new HNZ logo, in many different forms. So, keep an eye out, and let us know what you think.

Hemlibra approved

As of 1 October 2023. Te Pātaka Whaioranga – Pharmac has widened access to emicizumab (branded as Hemlibra) for the treatment of people with moderate and severe haemophilia A without factor VIII inhibitors. This is a fantastic development, and the result of plenty of lobbying by HNZ, and others, on behalf of its members.

As stated in the Pharmac proposal, this would mean that any person with severe haemophilia A, defined as endogenous factor VIII activity less than or equal to 2%, would have access to funded emicizumab, regardless of FVIII inhibitor titre. For those eligible, Emicizumab is administered at a dose of no greater than 3 mg/kg weekly for 4 weeks followed by the equivalent of 1.5 mg/kg weekly.

The Chair of Haemophilia New Zealand, Deon York applauded the announcement, saying: "This represents the most significant change to care and treatment for people with severe haemophilia A for nearly 25 years. We are elated by the decision and convey our heartfelt appreciation".

"Widening access to this treatment will help people manage a life-long bleeding disorder" Pharmac's Director, Pharmaceuticals Geraldine MacGibbon says. "We expect that 115 people will access this treatment during the first year of funding, increasing to 140 people as they transition from other treatments. There are many children in this group, so it is positive that they'll have a life-long treatment available to them."

Before approval of Hemlibra, people with moderate and severe haemophilia A have had to treat with regular intravenous infusions of factor VIII, often multiple times per week. Hemlibra is a vast improvement on this regimen because is given as a subcutaneous injection. This means that people, or their caregivers, will are able to administer the treatment themselves. Geraldine MacGibbon says.

"This decision has the potential to make a big difference to their lives. Through our consultation, we heard from a number of people living with severe haemophilia A and their whānau, who shared information about the difference this change will make for them."

As stated in HNZ submissions to Pharmac, access to Hemlibra will not only improve the lives of individual haemophilia patients, it is also likely to reduce the burden on the wider health sector by reducing the need for specialist consultations and reducing in the number of bleed events requiring hospitalisation.

Emicizumab is supplied by Roche Products (New Zealand) Ltd. Roche's General Manager, Alex Muelhaupt, says it's wonderful news that a wider group of New Zealanders can now access these treatments. "We want to acknowledge the work done by the haemophilia community over the years, leading to this agreement."

The 21st Australian Conference on haemophilia, vWD & rare bleeding disorders

HNZ outreach workers Darian Smith and Loren Silva attended the 21st Australian Conference on Haemophilia, vWD, and rare bleeding disorders in Melbourne in August. Prior to the conference they participated in the Australia and New Zealand Haemophilia Psychosocial Group meeting. Between them they attended as many sessions as they were able to, and reported back on each one.

ANZHPG meeting 23 August 2023

On 23 August HNZ outreach workers Darian Smith and Loren Silva attended the Australia & New Zealand Haemophilia Psychosocial Group meeting. This group includes our HNZ outreach workers, and the Australian social workers and clinical psychologists who work with people with bleeding disorders, much the same as our outreach workers do. One difference is that in Australia these professionals are hired by the hospitals as clinicians alongside other health staff, like haematologists, nurses, and physios, rather than by the Haemophilia Foundation of Australia. Each hospital has both a social worker and a clinical psychologist, however they do not focus on bleeding disorders full time as we do. Also, practice is divided between paediatrics and adult hospitals.

Some key points noted at this meeting included:

- Patients may need support with the new Hemlibra treatment barriers. Injection does sting, so it is a different kind of pain that people need to work through.
- Patients with extreme aversion to treatment have been trialled using Nitrous Oxide to relax during treatment.
- It is important to observe parents' emotional temperament when treating children, as this can often indicate why a child is having a negative emotional reaction to treatment.
- Aged care patients transitioning to residential facilities have anxieties about not receiving adequate care from nursing home staff.
- Resource sharing: One staff member has created story books and heart felt videos about individual patient's stories – something tangible that young patients can reflect on and show their friends and teachers to help normalise and destigmatise their bleeding disorder.
- Wellbeing workshops for clinical staff. Staff have identified long term mental health effects due to the difficulties of caring for patients with bleeding disorders. Social workers and psychologists conduct monthly wellbeing workshop for clinical staff to help alleviate these impacts.

Gene therapy

This was a pre-conference session.

Gene therapy is the next big thing in the treatment of haemophilia and has been for quite a while. In this session, one presenter said cure, while the others said it's not a cure. So, there are still differences between colleagues on topic. The haemophilia B gene therapy is currently more robust than that for haemophilia A. Viral gene therapy means that if patients choose to go this direction and it is unsuccessful, they will not be eligible for any future viral gene therapy treatments because they have already been exposed. Scientists are looking at non-viral gene therapy and gene editing in future.

When a patient receives gene therapy treatment, there is intense follow up and monitoring for 1 year following their injection. Patients may need to be on steroids to suppress immune response during this 1-year phase, which can cause other negative symptoms.

Clinicians advise that haem B patients will more than likely have no bleeds for 20 years +, whereas currently haem A evidence shows that bleeds will only stop for 5-7 years and then will slowly come back.

A key aspect of this process is the World Federation of Hemophilia's shared decision-making tool: https://sdm.wfh.org/. This web-based process helps patients to evaluate all aspects of this major decision before agreeing to be on a clinical trial or being treated with gene therapy.

Plenary One - Treatment landscape into the future

Gavin Finkelstein – from then to now.

In the early days, treatment was whole blood & hospital stays. Cryoprecipitate changed things, as infusions became quicker and easier. Freeze dried plasma product freed things up even further and travel was now possible (with planning) and patients could store small amounts in the fridge at home. Supply was an issue though, and more debilitating damage may have occurred as a result of these restrictions.

The infected blood scandal in the 80s lead to many treatment-based HIV and HCV infections. Many people have undergone blood treatment to remove those viruses.

The next phase of treatment were the recombinant products, as well as effective treatment for HCV. These are now available for everyone. 2018-19 brought extended half-life products.

Hemlibra now available and is life changing. This treatment requires fewer injections, is easier to deliver, and has a beneficial impact on quality of life. There are more products like Hemlibra to come.

Next comes gene therapy. Due to extensive trials, we know that it works in most cases. Cost benefits are currently being assessed.

Dr Glenn Pierce: What is precision or personalised medicine? New and emerging treatments for all bleeding disorders.

Ultra extended half-life products have been approved in Europe and US but not yet here. Gene therapy should take us to close to normal levels of factor. Rather than peaks and troughs, non-factor products and gene therapy should provide a steady level.

Extended products mean longer between treatments while maintaining protection.

Emicizumab is now approved, and Efanesoctocog Alfa was recently approved and provides higher trough levels, e.g. weekly treatment with a peak over 100% and trough of 15%. Emicizumab provides 15% equivalence. This basically turns severe haemophilia A into mild.

Emicizumab takes on one of the roles of FVIII, that of bringing FIX and FX together. It achieves zero bleeds for many patients.

Efanesoctocog Alfa is a new product. It protects FVIII from being taken out of circulation. In some places it is being offered as an alternative for Emicizumab.

There are many factors to consider before deciding on the right treatment for each individual. These include lifestyle, bleeding phenotype, joint status, treatment adherence, and venous access. Gene therapy requires more consideration. It has a lifelong treatment impact whether it works or not, so it is important to evaluate the benefit/risk, cost, etc. The journey for deciding the path through treatment options should be a shared decision making process with patients and clinicians. WFH has developed a shared decision-making tool on their website.

There are still many inequities in the world of haemophilia. Much of the world has little or no access to treatments. VWD and rare bleeding disorders haven't shown as much progress in treatment options. Women with bleeding disorders have been historically overlooked. Treatment for inhibitors has improved with Emicizumab for haemophilia A but not for haemophilia B. Orthopaedic access can be challenging for joint replacement. Clinical trial access is not available in much of the world. However, work is being done to address some of these inequalities. Gene therapy may provide significant relief for lower income countries that cannot sustain other treatments and prophylaxis.

What's next in treatment?

Proteins and RNA rebalancing agents, including anti-TFPI antibodies (marstacimab, concizumab), and SerpinPC, and fitusiran, which is RNA given once a month. Gene therapies, including next generation AAVs, non-viral gene therapies, and gene editing.

The goal is a functional cure.

Clare – Hopes for the future: reflections from a parent.

Clare is a mum of two boys who have severe haemophilia A. Prior to their birth, there was no family history of bleeding disorders. First son diagnosed at 6 months old. Bruising from kicking in the high chair led to diagnosis at the ER. A port enabled the family to travel. Switching to Emicizumab cut 3 port infusions a week to one subcutaneous treatment a fortnight. Such a huge change, and improvement in quality of life.

Second son was diagnosed at birth, so treatment started early. He has had no bleeds, no hospitalisation, and no port. It has been a much smoother journey.

The ideal would be a treatment without needles. Gene therapy provides hope. However, the current treatment provides a life without bleeds, which is wonderful.

The real hope for the future is eradicating it entirely from

future generations. Want kids to be able to live their lives without worrying about haemophilia.

Plenary 2 – Gene Therapy

Dr Glenn Pierce – Overview

Gene therapy represents a one and done cure, but we aren't there yet.

For gene therapy treatment, an inert virus is infused with the corrected gene. It is infused and travels to the liver and then into the nucleus, where it sets up stable DNA that makes the FVIII or FIX protein. Factor IX is secreted well, but factor VIII isn't. The FVIII therapy has a variable therapeutic response, with a year-on-year loss over 6 years. The half-life is about 123 weeks. FIX has a variable response but maintains stable levels over time and is projected to last at least 25 years.

Many gene therapy trials have taken place across the higher income countries in the world. Two are approved in the US and Europe. One for Haemophilia A and one for Haemophilia B. There will be further products to come through that will improve upon them.

Patients have hope. Gene therapy is working but not perfect. It is potentially cost effective. The question is whether to do it now or to wait for an improved version.

Also, what happens if it doesn't work, and is it worth it if you have to do all the monitoring for months after?

Jane Portnoy – Psychological overview

Gene therapy marks a substantial shift in the entire experience of haemophilia. There are challenges, however, and it doesn't always work, or you may not be eligible. Finding out it didn't work, or you are ineligible can bring on significant grief.

Even successful gene therapy comes with challenges, loss of identity, complications, etc.

The first challenge is making the decision to participate. There are a range of resources to help patients figure out what questions to ask and how to make their decision about engaging with gene therapy or not. Some patients have poor health literacy or unrealistic expectations, and the science is complicated. There is no lived experience or advice from those who went before you to draw on.

Participating requires a range of lifestyle adjustments – no alcohol, no unprotected sex, contraception adhered to until sperm clear, significant follow up and testing requirements. Even when agreeing to these, it can be hard to follow through, especially for young people with many life pressures.

Having a chronic illness increases the risk of mental health challenges, and the stress and pressure of going through this process and the significant risks, changes, and commitments will have an effect. Psychosocial assessments and interventions are vital. It is important to have a support system in place.

Inter-generational trauma within the bleeding disorders community is significant, and some patients will be hesitant, or will find their feelings from the bad blood era are triggered by a new treatment with unknown risks.

Following gene therapy, steroids may be required to assist with reactions. Steroid side effects can have significant impact on mood, sleep, weight gain, acne, etc. This can impact relationships, jobs, health, and/or family members. Social workers, counsellors etc. are vital to help manage these issues.

Identity can shift drastically when you have spent your life as a person with haemophilia and you suddenly don't have the same thing, it can impact identity and connection to your specialised community.

Gene therapy treatment needs a wrap-around support to ensure patients come through it safely and having experienced the best care possible.

Concurrent 2: Rare bleeding disorders

Chauntelle – personal experience, FXIII

Diagnosed at 20 when her tonsils were removed. She took multiple tests to explain her continued bleeding. Both parents were also tested and were found to be carriers, but symptom free. When Chantelle was younger her bruising was questioned, but no one knew the condition. It was hard to explain the bruises as people didn't understand.

At 21 she had her wisdom teeth removed and was infused with FXIII ahead of surgery. Subsequently, she had no post operative bleeding.

Chantelle had twins, and needed infusions throughout pregnancy. She also experienced a bleed which caused a stroke and seizures.

She now has FXIII infusions every four weeks.

Dr Sally Campbell – Rare Bleeding Disorders

There are many rare bleeding conditions, and a clinician may only ever see one or two of a condition. Rare is hard to quantify but could be one in a million or two million. Rarest has less than 30 in the world.

The clotting cascade is very complex and detailed, which is

why people can present differently with the same condition.

The size of an injury has an impact as well. So does the quality of the artery, cholesterol level etc.

Rare bleeding disorders have a wide variety of symptoms. People with the same factor levels might present very differently. Typical symptoms are mucosal tract bleeding and excessive bleeding following procedures.

Severity classification is difficult with the following disorders, because there are so few with a specific condition to study:

- Factor I (Fibrinogen): Could be not enough, or enough but poor quality. Can present in infancy with catastrophic bleeding. Treatment needs to be tailored to the patient. There are three kinds of fibrinogen concentrates available to use for treatment.
- Factor II (Prothrombin): Autosomal recessive. If you make none, it's not survivable. In severe clinical settings there are treatments such as FFP, but too much can cause clotting.
- Factor V: Autosomal recessive. Frequent symptoms are epistaxis and heavy menstrual bleeding. The first concentrate treatment is being developed. Current options FFP and platelets. Platelets engulf and release FV where it's needed.
- Factor VII: More common of the very rare disorders. Autosomal recessive. Huge range from lethal to mild to asymptomatic. Can be heavy menstrual bleeding or haemarthrosis or CNS bleeding in around 16%. Treated with Novoseven, which has a short half-life but can be used as prophylaxis and get improvement even though it's out of their system quickly. A longer acting FVII concentrate is coming, which looks promising. Sometimes women who don't usually have clear symptoms will haemorrhage with childbirth.
- Factor X: One of the most severe. May present with umbilical bleeding, CNS, or GIT. Postpartum bleeding in carriers common when they don't bleed normally. Two concentrates and a better version of FX coming in the future. Difficulty getting treatments to market because there are so few patients.
- Factor XI: Second most common of rare disorders. 1 in a million for most, 1 in 450 in Ashkenazi Jews and French Basques. Varied presentation. Asymptomatic to bleeding. May bleed post-surgery. Treatment is nothing, TXA, FFP, or plasma derived product.
- Factor XII: delayed wound healing, recurrent miscarriage. Factor often consumed in inflammatory processes so looks low when it might not be a genetic reason. Treatments Novothirteen. Aiming for around 15% for prophylaxis. Use prophylaxis during pregnancy.
- VKDCFD: Autosomal recessive. Affects vitamin K metabolism, so it's like warfarin every day. High dose vitamin K can help, or factor.

Jenny – personal experience, acquired haemophilia

First symptoms: Got bruises for a while. Then a lump behind the knee and bruise. Went to doctor and got blood test and ultrasound of knee. Got a call from the doctor saying come to emergency immediately. You could have a stroke any minute. Passed blood clots in toilet. No previous issues or family history. Scary but appreciated the medical team. Body rejected FVIII and was treated with FVII to trick the body into reaccepting FVIII. Now in remission. Acquired haemophilia happens to 1 in 1.5 million people. Still on 6 monthly checks. Risk of recurring but the longer it goes without the better. Three years is the biggest risk of recurrence.

Dr Jane Mason – Acquired haemophilia

Emicizumab can be a useful off-label treatment for this.

Rare, life threatening, can be fatal.

The body develops antibodies against their own FVIII.

Causes can be autoimmune, malignancy, infection, pregnancy, medication – but half or more there is no cause identified. But the majority of patients can be treated to remission.

1.5-2 cases per million each year. More common in elderly with median age 74 yrs. Small peak in pericardium women

20-40 yr old.

Very different to congenital haemophilia. Diagnosed later, no genetic pattern, can effect both genders equally, not usually joint bleeds, no correlation with factor levels and bleeding levels. Mortality is increased.

Time from bleeding event to diagnosis can a number of days or even up to a month depending on awareness in local doctors etc.

Management – control bleeding, treat underlying disease, eliminate inhibitors.

Risks - some patients die from bleeding or from the immune suppression treatment complications.

Simple measures critical. Rest,ice, compression, elevation. Avoid further tissue trauma.

Bypassing agents given, replacement therapy, Emicizumab seems to be effective but not licensed for acquired haemophilia.

Hereditary Hemorrhagic telangiectasia – Alex Klever

Uncommon inherited genetic disorder that causes development of abnormal blood vessels.

Autosomal dominant

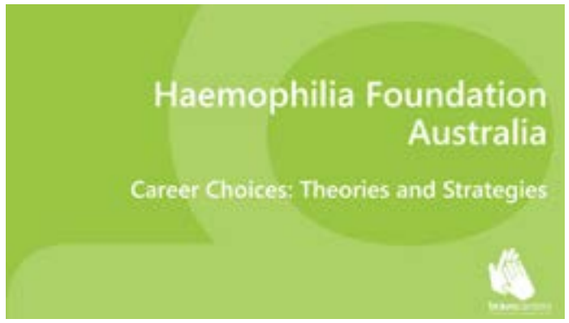
Often family history. Nosebleeds, little red dots, nose bleeds, gastrointestinal bleeding, anaemia, and AVMs.

Tranexamic acid useful to treat with. Humidification, nasal sprays, ice pack on the back of the neck all useful. Get an ENT specialist to consider options.

May need iron infusion. Endoscopy can be helpful to find active bleeding.

Management – iron supplement, blood transfusion, AVM screening – MRI.

Concurrent 3: Making career choices



Many people with bleeding disorders have experienced discrimination in the workplace. There are two ways employers may discriminate against people with disabilities:

Direct discrimination – treating employee unfavourably because of an attribute.

Indirect discrimination – imposing unreasonable requirements and making job unfairly difficult.

People with disabilities need to think about their career values, motivations, and career interests. It is important to understand your learning style; whether the way you learn best is visual, auditory, or kinaesthetic.

There are some key ways in which you can work towards employment success:

- Set goals. Having a 5-year plan will help to keep you focused. Ask yourself:
  - What do I need to accomplish?
  - Who can help me?
  - When will I get this done?
- Always have a bias towards action. Making regular baby steps towards your goal will help maintain momentum.

There are some interesting psychological concepts related to career development:

Design thinking model - Design thinking is a non-linear, iterative process that teams use to understand users, challenge assumptions, redefine problems and create innovative solutions to prototype and test. Involving five phases—Empathize, Define, Ideate, Prototype and Test—it is most useful to tackle problems that are ill-defined or unknown.

Hope action theory - This theory holds Hope as the centre point of career development. Associated with hope are competencies such as self-reflection, self-clarity, visioning, goal setting and planning, implementation, and adapting.

Core values for both of these theories is self-exploration, self-acceptance, and constructive action.

There is polarising commentary on whether it is appropriate to disclose your BD to potential employers. Some said 'yes', because worst case scenario someone is aware, some said not because there will always be inherent discrimination. Either way, it is important to be educated about your BD so that you can confidently discuss it is required.

Plenary 3: Women and girls with bleeding disorders

Women are chronically underdiagnosed, and the issues related to their BDs are misunderstood. In women, BDs can cause other disorders. For example, bleeding in the ovaries can cause endometriosis. Women with BDs have unique issues and usually need other clinicians like gynaecologists involved.

At this session, a women with vWB type 3 told her horrific story of many comorbid conditions, and another with mild haemophilia A, who has two sons with severe Hem A told her story.

When you undertake gynaecological training you are not taught how to test for BDs, only told to check if someone presents with extremely heavy period. However, one study found that 11% of women who present to specialist with extreme menstrual bleeding have a BD. As vWB factor increases when people are stressed, rendering tests inaccurate, it is believed that this statistic understates reality. Experts say not to test women when they are stressed, although the act of testing can induce stress in many women.

Women with heavy periods are more likely to have endometriosis. In today's society, women are having more periods which the system is not designed to treat. While contraception reduces bleeding, intrauterine devices like Mirena don't talk to ovaries, which is where more BDs occur.

A couple of useful resources were mentioned:

https://letstalkperiod.ca/ Self diagnosis and resources for women with BDs

https://www.howcollaborative.org/ A group of clinicians dedicated to helping women with healthcare concerns.

Plenary 4 – Mild Haemophilia

Diagnosis and treatment – Dr Heather Tapp

Generally speaking, mild haemophilia is defined by factor levels between 5% and 40%. However, it is hard to definitively pinpoint moderate or mild as there can be variation between labs and types of tests. As you age factor levels tend to increase. Also, other modifying genes may be impacting the bleeding phenotype. It is more common today that women, who were previously coded as carriers, are being included as mild haemophilia patients.

Mild diagnosis is often delayed. The average diagnosis time is about 28 months, but it could be five years or more. This is compared to 5.8 months for severe and nine months for moderate. Some people go undiagnosed.

People with mild haemophilia are often less engaged with the HTC. They are usually not on prophylaxis and since they have infrequent spontaneous bleeds they are less experienced at recognising and managing bleeds. They don't have home treatment and come in only when they think the bleed needs intervention and often present later than ideal for a bleed treatment.

Canadian study looked into why people don't recognise bleeds and access treatment. Some in the study didn't know their baseline factor levels. Many felt much of the education about haemophilia doesn't apply to them because they are "only mild" and employed a wait and see approach to possible bleeds. Following the study they developed an app called HIRT (Haemophila Injury Recognition Tool). Helps to self manage and assess.

The international Dynamo Study group did a study with males with non severe haemophilia. They found that joint status is still an issue for mild haemophilia, perhaps even more than in those with higher severity.

So, what does the future hold?

There are currently unmet needs for people with mild

haemophilia. There needs to be more support for patients, and greater engagement with HTCs. It is important to recognise the need for prompt treatment of bleeds, and the effect on joints of delayed treatment. Consideration should also be given to access to novel therapies.

Medical issues – Dr Stephanie P'ng

Most people with mild haemophilia are not on prophylaxis. Prophylaxis is to prevent joint damage but there is evidence of joint damage in mild haemophilia, showing there have been bleeds not treated properly even if not noticed or acted on. A Canadian study found that the maximum joint score for people with mild Haemophilia A was actually higher than those with moderate or severe, due to lack of appropriate treatment.

Inhibitors are still an issue for about 4-8% of mild haemophilia patients and can result in a more severe bleeding phenotype.

Risk factors for inhibitor development include family history, age, and mutation type.

Treatment options are replacement, Emicizumab etc. Although this difficult to get funded.

One challenge is improving bleeding disorder knowledge in patients as well as in healthcare workers. Low knowledge can mean risk behaviours are not discussed, and related bleeding episodes are not manged properly. Minimising Haemophilia in women can lead to mismanagement of menstrual bleeding and pregnancy related bleeds. The lack of engagement by mild patients in their local haemophilia community can result in a lack of support.

The most common treatment recently has been via factor replacement with standard half-life then extended half-life products. The latest treatment is Hemlibra (emicizumab).

Inhibitor development is still an issue for people with mild haemophilia and has been put at 4-7.8% over ten years. This usually follows intensive treatment, perhaps for surgery or dental treatment. This can result in spontaneous and persistent bleeding not previously experienced.

Issues with treatment include difficult venous access and inhibitor development, particularly following surgery.

Alex Klever – Nursing management including surgery in mild haemophilia

It is important to know the type of haemophilia you have and what your normal factor level is, and to have a good relationship with your HTC. Visit the HTC team regularly, along with regular GP and dentist visits. Tell family and other health care professionals about your diagnosis.

If you are undergoing surgery, it is essential to let your HTC know. This allows them to plan appropriately, and to keep all parties informed. If you have an injury, it's important that you have a basic level of first aid knowledge. This includes recognition of a bleed, an understanding of the RICE process, and knowledge of the presentation pathway at your HTC. If you have treatment at home, treat AND call your HTC. Also, make sure that you stick to your rehabilitation plan to prevent ongoing issues or a re-bleed.

Abi Polus - Physical activities and sport across the lifespan

Studies comparing milds to age matched non-haemophilia people showed joint dysfunction, pain and disability increasing with age, and a lower quality of life. They also missed 3-5 days' work a year due to their haemophilia.

We rarely see bleeds in mild children, other than sporting injuries, In adults bleeds occur around sport, repetitive tasks, and farming injuries. Many milds lack the ability to identify bleeds. They may also lack the ability to self-infuse. Because many people are not diagnosed until later in life, they lack the awareness and support systems other people with bleeding disorders may have.

While their risk of bleeding may be less than those with a greater severity, there is a concern that patients with mild haemophilia may underestimate their actual risk, leading to delayed treatment and significant consequences.

Previously, sports suitability for milds has been categorised by the probability of collision and/or the historic incidence of injuries. These days more complex parameters are being proposed. These include biomechanical aspects, level of difficulty, and risk of injury.

Other factors to consider when selecting a sport are the availability of treatment in the country/location you're playing it, as well as your joint status and fitness level. Age is another key factor to consider. As children grow older the amount of contact allowed in many sports increases. Adolescents have times of increased growth, when they are particularly vulnerable to injury due to an imbalance between strength and flexibility.

The majority of literature now advises that decisions to participate in sport are individualised and are made collaboratively taking into account clinical status, the patient's interests and desires, and their capabilities. All sporting choices should be informed decisions.

Genetics, inheritance, and family planning

A mutated gene that leads to a bleeding disorder can stem from an abnormal protein or from less or no protein within the gene. Low factor VIII means haemophilia A, low factor IX means haemophilia B, and low von Willebrands factor means von Willebrands disorder.

The vWB gene produces vWB factor which helps with platelet plug and some factor production. Whereas low factors VIII and IX interrupt the clotting cascade, meaning the clotting process never finishes.

The FVIII and FIX genes are found on the x chromosome, which makes haemophilia rarer in women because they need two faulty copies. However, vWF is located on chromosome 12, so vWD equally impacts men and women.

The screening for gene alterations that cause BDs is not accurate, current testing analyses general population. Tests won't look at specific BD genetic alterations, so you're better off having a targeted gene test. Testing before pregnancy is ideal. You can test during; however, it does take a long time.

Discovering gene difference can lower self-esteem.

HFA has a resource on their website about how to have a conversation about disclosing genetic status to potential reproductive partners.

Your outreach team

Haemophilia NZ has four outreach workers, who care for the psychosocial needs of our members. They came from a variety of backgrounds, and are focused on making sure that your needs are met. They are based in each of the four regions: Northern, Midland, Central, and Southern.

Darian Smith: Northern outreach

I've been with HNZ for 2 years now and it's absolutely flown by but it's been wonderful to meet so many of our members and learn about their journey with bleeding disorders. There are still a few I haven't managed to connect with face to face so if you'd like to catch up, just get in touch. I grew up in Auckland so I know the region pretty well. I currently live a short distance from the Waitakere Ranges with my wife of 23 years, Adrienne, and our black and extremely fluffy cat, Athena. Athena was named after a Greek goddess and I think she's taken that to heart.

Adrienne and I both share a passion for writing. She writes contemporary romance and I the author of a series of murder mystery mixed with epic fantasy called Agents of Kalanon. The first of the Agents of Kalanon series was a finalist for a global award and my writing has also won local awards such as the Koru Award and the Sir Julius Vogel Award.

I've also done some TV work as an extra and you can watch me get murdered by Spartacus if you're particularly eagle-eyed and find the right episode.

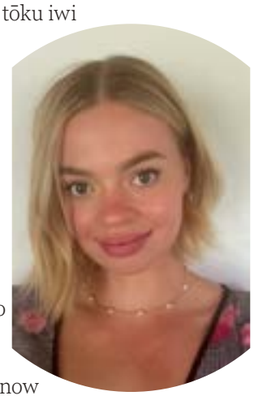
After training as a counsellor and family therapist I started a free counselling clinic through my local CAB and worked in private practice for several years. I also spent time working for Arthritis NZ and, when my wife was diagnosed with a form of muscular dystrophy, I took up an Outreach Worker type role with the Muscular Dystrophy Association of NZ. I worked there for 11 years and learned a great deal about genetic disorders which has been relevant for my work with HNZ. As Adrienne's condition has progressed, however, we decided that a change in my work focus would be wise and so I joined the HNZ team.

I've loved being part of the HNZ family and seeing the amazing progress that's been happening with treatments and improving the quality of life for people living with bleeding disorders. It's a privilege to be part of it. I look forward to getting to know you all even better as time goes on.

Loren Silva: Midland outreach

Tēnā koutou,

Ko Maungatautari tōku maunga
Ko Waikato tōku awa
Ko Tākitimu tōku waka
Ko Ngāti Kahungunu o te Wairoa tōku iwi
Ko Whaakirangi tōku marae
No Kemureti ahau
Ko Loren Silva tōku ingoa



I have been working with HNZ for over a year now and it has been great getting to know and support members within the Midlands Region. Midlands has had a difficult few years, with covid and the high turnover of staff within HNZ and the Waikato Haemophilia Treatment Centre. I feel that 2024 is going to be a fantastic year for Midlands as we now have a solid and reliable team ready to support our members and put on some great events. There is still the need for Midlands members to volunteer their time to assist in the planning and execution of our regional events, so if you are interested in this please feel free to contact me.

I was born and raised in Cambridge, 20 mins outside of Hamilton Waikato. I currently live here with my partner and our Pug named Spud. He is such a character and is a trained therapy animal. Spud loves going to the local rest home to get cuddles and treats from the residents.

I am not far off from becoming a Psychologist, with only 1 year of study left before I can apply for my registration. However, after 5 years of study, HNZ has been an opportunity for me to have a break from study and focus on enhancing my practical experience in a social service environment. My future career goal is to become a registered Psychologist that supports Māori mental health initiatives.

I love the beach, the gym, reading, gardening, painting, attending concerts. My partner and I recently went on a trip to Thailand, giving me a case of the travel bug!

It has been such a great experience working with HNZ, and I am looking forward to what lies ahead for this community of awesome people.

Lynne Campbell: Central Outreach

I have worked with our HNZ families as the Central outreach worker providing education, support and advocacy since April 2009 from my home based office, currently in Horowhenua. This location has proven to be ideal given its proximity to both the Wellington HTC, and the Palmerston North HTC, the two large haematology centres located in my region.

My background is in in biology and science teaching, as well as educational guidance and counselling where I worked with a diverse range of student groups and enrolment categories, including adult students and students enrolled for health and medical reasons.

With a keen interest in genetics, a science background and transferrable guidance skills, I have redirected my passion from working with students to helping people of all ages with rare inherited bleeding disorders.

In the time I have worked in HNZ, treatments have improved out of sight. To see this change is so heartening. We are entering an era where young people with bleeding disorders really can live well with their condition.

Implications for women are well recognised. Although there is no right or wrong decision, reproductive options are discussed openly now.

The importance of physiotherapy and exercise are well documented. These have become an important part of joint bleed rehabilitation.

I enjoy my work with our HNZ families, it is a privilege to work with you. We outreach workers not only learn so much from you, but we do get attached to our families over time!

It is so important that we do not lose sight of where we have come from though. HNZ membership ensures we have a voice and representation should unforeseen developments occur and as new treatments become available.

Outside of HNZ work, my interests include family, gardening, art, fashion and music.

At community level, I am a volunteer for Riding for the Disabled and a member of Rotary, which involves contributing to a range of community based projects in the region.

Do please keep in touch, let me know if your contact details change and please let me know if you would like to catch up.

Vic Turner: Southern outreach

My name is Vic Turner (Vicki if you are my mother).

I currently live on a 50 something hectare block of land divided in to grazing for the horses, gorse (native nursery) and regenerating bush. There is an utube doco on a person called Hugh Wilson, which may interest any eco warrior's only fools and dreamers. My husband Burt and our youngest who is 12 live in an old 1900 shack, which shows the ravages of Earthquakes, rain, and wind events. We love it.

I've lived here now about 20 years and have worked as a

Waitress (late starter 35)

Qualified as a counsellor

Worked in transitional women's housing chch

Specialised in equine assisted psychotherapy

Had contracts with men and trauma and I am hope.

And still volunteer for a group supporting women with baby loss

Prior to this, I trained as an enrolled nurse at Burwood hospital, worked at Templeton hospital and Te Poutama arahi rangitahi.

So you have probably guessed by now I am not 27!!

I really love my new job and am striving for the perfect work. Life balance.Im always grateful for the challenges that come my way.

Having completed my first outreach I am grateful to those of you who shared your journey with me, it certainly gave me a sense of purpose and direction. I have recognised the need to develop positive relationships with the medical profession and of course the members. I am currently compiling lists of ideas relating to supporting, educating and advocating. Therefore, if you have any ideas they will be grateful received. With Christmas on the horizon, I have enlisted some enthusiastic members to help organise end of year celebrations in every region. I am also head hunting for committee members so if you want to help give me a shout out.

Around the branches

HNZ's Branch committees enable all our members to participate in the running of the organisation, and to connect with and support one another. Each Branch runs a number of events through the year, to help educate their local members, to make sure that support goes where it's needed, and to have a little bit of fun. Here's what they've been up to recently.

The **Northern** branch has had a fairly busy second half of the year. After the lows of Covid, it's lovely to see this group prioritising member activities. At the end of July the team had a fantastic outing to Butterfly Creek. There was a very good turn out, and everyone loved the opportunity to get out and see something a bit different. Of particular note was the crocodile feeding! The Northern whānau enjoyed an adrenilin filled outing to Game Over Auckland in October. There, members were able to take the go-karts for a spin, battle it out at laser tag, and for the more sedate among us, shoot a round of minigolf. At the end of November the crew gathered at Goode Brothers New Lynn for a spot of Xmas lunch. It was lovely to get together before the chaos of Xmas was fully unleashed, and a nice relaxed way to wind up the year.

welcome dinner for the new outreach worker Vic. It was great to get the crew together anda show Vic how excited we all are to finally have a full staff complement. Following her dinner, Vic was off on a tour of the region. Feedback was very positive, and everyone loved the chance to lay eyes on her. On the back of this trip, there seems to be some momentum growing again down south, and group of volunteers are putting their heads together for future events. The first of these was the Southern end of year pool party. Members gathered at Te Hāpua Halswell Summer Pool for an afternoon of chlorine scented fun, before enjoying a lovely festive dinner.

The **National Youth Committee** held a hui in Wellington in September. This was well attended, and everyone had a great time. The event mixed a bit of education and planning, a bit of socialising, and some go-karting. They're looking forward to their sailing event, which is all set to go in January.

Thing were a little quieter in **Midland**. Outreach worker Loren planned a members' dinner in Tauranga, but turn out was low. Still, those in attendance had a nice time. For their Xmas outing the crew went to the Great Kiwi Summer Festival at Lake Karapiro. This is a fantastic event, with fun things to do for the whole family, and the Midland members had a great time winding down the year.

Central held their AGM in September, at the Herb Farm Café in Ashurst, just north of Palmerston North. They had an excellent turnout for lunch, followed by the AGM, and the opportunity to check out the stunning gardens and have a go at counting how many themed gardens there were (the answer was 14!).

In mid-November, members from the Central region visited Staglands Wildlife reserve for their Xmas event. They were able to arrive when they saw fit, then wander around admiring the animals, before gathering for a BBQ lunch on the deck. This was a fine way to finish up the Central year.

Down **Southern** way, this end of the year began with a

Latest news

Assessing Reasons for Treatment and Treatment Type in Females with Bleeding Disorders

By Patrick Daly

December 15, 2023

A majority of female patients with inherited bleeding disorders, including von Willebrand Disease (VWD), hemophilia A, and hemophilia B, experienced bleeding symptoms that may call for the initiation of prophylaxis with factor and non-factor replacement therapy, particularly with more than one or two bleeding events per year, according to a retrospective single-center database analysis.

Additionally, during a presentation at the 65th American Society of Hematology Annual Meeting & Exhibition in San Diego, California, lead author, Daniel Barnett, MD, from the Banner University Medical Center in Phoenix, Arizona, suggested that "heavy menstrual bleeding, joint bleeding, joint pain, [and] gastrointestinal and genitourinary bleeding are reported in this patient population as reasons to start prophylaxis with hemostatic agents."

The analysis reviewed 101 female patients from the Banner MD Anderson Cancer bleeding disorder database, of whom 64 had a diagnosis of hemophilia A, hemophilia B, or VWD. Researchers reviewed the type of treatment, reason for treatment initiation, and type of administration.

Overall, 13 of 17 (76%) patients with hemophilia A were receiving prophylaxis with factor replacement or emicizumab. The most common reasons for initiating prophylaxis were joint pain and bleeding in six patients and menorrhagia in three patients. Among the four patients with hemophilia B, two (50%) were receiving factor replacement, with rectal bleeding and menorrhagia recorded as reasons for starting prophylaxis.

In the VWD group, 24 of 43 (56%) patients were receiving prophylaxis with one or more of the following: factor replacement, desmopressin, or antifibrinolytic replacement. The most common reason for initiating treatment was menorrhagia in 13 patients followed by joint bleeding in four patients. Additional reasons including trauma, mucocutaneous, urinary tract, and pregnancy bleeding.

Notably, patients with VWD were much more likely to require central intravenous treatment administration via a chest portacath (n=14; 33%) and were more likely to have received their diagnosis prior to age 18 years (n=16; 37%).

In closing, Dr. Barnett noted, "Once factor replacement is started, patients are pleased with the change in their health status after initiation of treatment and continue to be compliant with treatment recommendations although they may have difficulty in administering those treatments."

Reference

Barnett D, Nance D. a retrospective analysis and characterization of female patients with bleeding disorders hemophilia B, hemophilia B and von Willebrand's disease and their treatment type at Banner MD Anderson Cancer Center. Abstract #1254. Presented at the 65th ASH Annual Meeting & Exposition; December 9-12, 2023; San Diego, California.

Source: https://www.docwirenews.com/post/assessing-reasons-for-treatment-and-treatment-type-in-females-with-bleeding-disorders

Haemophilia A: new data on therapy in newborns

December 18, 2023

Emicizumab is well tolerated and can control bleeding even in the youngest children, starting from birth. These are the new results on the monoclonal antibody used against hemophilia A that arrive from San Diego, a few days after the close of the American Society of Hematology (Ash) congress. An additional wealth of knowledge that comes specifically from the primary analysis of the phase III HAVEN 7 study, and which acts as a corollary to the recognition Of Takehisa Kitazawa e Johnny Mahlangu with the Ernest Beutler Lecture and Prize for drug development.

Hemophilia A in children

Hemophilia A is a rare disease characterized by bleeding, which is in turn due to the lack of a clotting factor, in this case factor VIII. There are several shape, more or less serious depending on how much the missing clotting factor is present (which can be present in variable percentages). Emicizumab is a bispecific monoclonal antibody, which compensates for the absence of factor VIII by acting as a bridge between factor IX and factor to be started in the first year of life, as explained by the experts. "Prophylaxis with factor VIII replacement therapy often begins after the first year of life due to problems related to venous access," he underlined Antonio CoppolaMedical director at the Haemophilia and Congenital Haemorrhagic Diseases Hub center of the Parma University Hospital, on the sidelines of the preliminary results presented at the same congress last year.

And give that HAVEN 7

Those arriving today from San Diego are helping to redefine the possibilities of using the drug in the little ones, which can also be administered subcutaneously, from birth, at different dosage frequencies. The new data coming from HAVEN 7 – conducted on cases of severe hemophilia A without factor VIII inhibitors, previously untreated or minimally treated – show that more than 50% of patients who received it (55 in total) had no bleeding requiring treatment. 16.4% had no bleeding, treated or untreated, and in total there were 207 bleeds in 46 patients, almost 90% due to trauma. There were no spontaneous bleeds requiring treatment in any patient.

Redefining the standards of care for hemophilia A

"The results of the primary analysis of the HAVEN 7 study presented at the ASH 2023 conference are extremely promising for the management of haemophilia A without inhibitors in newborns. The efficacy and safety of emicizumab in this age group, with significant control of bleeding, represent further confirmation of what has been seen so far in the emicizumab clinical development program and in clinical practice", he commented Flora Peyvandidirector of the Angelo Bianchi Bonomi Hemophilia and Thrombosis Center and head of the Complex Structure (SC) of General Medicine – Hemostasis and Thrombosis at the IRCCS Ca' Granda Foundation Ospedale Maggiore Policlinico in Milan, and director of the Department of Medical-Surgical and Transplant Pathophysiology of the University of Milan.

Peyvandi reiterated the importance of flexible administration thanks to the drug, given the difficult venous access. Furthermore, according to some studies, early prophylaxis improves long-term outcomes and reduces the risk of intracranial hemorrhage. "These results – concludes the expert – reinforce the importance of starting prophylaxis as soon as possible to improve long-term results, also reducing the emotional and physical burden for patients and their families. We cannot but welcome these advances which will significantly contribute to redefining the standards of care for our patients with haemophilia A."

Source: https://www.breakinglatest.news/health/haemophilia-a-new-data-on-therapy-in-newborns/

Uptake of new hemophilia gene therapies slow as field assesses options

By Deena Beasley

Dec 15, 2023

High cost, logistical issues and the prospect of potential treatment advances are holding back adoption of the first gene therapies for hemophilia, experts said this week during the American Society of Hematology's (ASH) annual meeting.

Experimental options discussed at the San Diego meeting included personalized treatments and next-generation gene therapies, many still in the earliest stages of testing.

People with hemophilia have a fault in a gene that regulates production of proteins called clotting factors. This can cause spontaneous bleeding as well as severe bleeding following injuries or surgery. Since the gene is carried on the X chromosome, hemophilia is almost entirely a male disease.

U.S. regulators over a year ago approved the first one-time gene therapy for hemophilia B, CSL's (CSL.AX) Hemgenix. Its list price of $3.5 million makes it the world's most expensive drug. BioMarin's (BMRN.O) Roctavian, the first gene therapy for the more common hemophilia A, was approved in June and has a U.S. list price of $2.9 million.

"The problem is nothing is perfect with gene therapy," said Dr. Margaret Ragni, professor at the University of Pittsburgh's hematology/oncology division. She said many patients are not great candidates due to underlying conditions such as having antibodies to blood factors, antibodies to the virus used as a vector to deliver the new gene, liver disease, HIV infection or obesity. The durability of the replacement genes and risks of long-term side effects are also open questions.

Dr. Michael Recht, chief medical and scientific officer at the National Bleeding Disorders Foundation, said gene therapies can be "transformative" for the right patient, but large medical systems have been stymied by their expense and logistics. "Three and a half million bucks is a lot to have sitting on your books, waiting for an insurance company to get around to cutting that check," he said. "In the entire Northeast region so far there hasn't been a single vector delivered yet."

BioMarin declined to comment on timing for commercial use of Roctavian in the United States, but said many hemophilia treatment centers have begun site preparation.

The first commercial patient was treated with Hemgenix in June. CSL declined to comment on use to date, but has said it expects around 50 commercial patients globally by next June. Bob Lojewski, CSL's North America general manager, said "a lot of patients" have been referred for Hemgenix and are waiting to get the therapy administered.

Sources said the hemophilia community has been hesitant to embrace gene therapy in part because in the 1970s and 1980s thousands of patients became infected with hepatitis C or HIV from donated blood.

A major advance came in the early 1990s, when bioengineered clotting factors replaced those derived from blood plasma. About 10 years ago, long-acting factor replacements were developed, greatly reducing the number of infusions needed to prevent traumatic bleeding.

Both Hemgenix and Roctavian deliver replacement genes by an infusion to the liver using a modified virus. Afterward, patients develop antibodies to the virus, making them ineligible for any future gene therapies that use the same delivery system.

'Incredible Innovation'

Hemgenix data presented at ASH showed that after three years, 51 out of 54 trial participants remained free from the need for regular factor replacement infusions, although nine needed treatment for liver enzyme issues.

Roctavian trial results showed that after three years, 46 out of 134 patients had factor VIII levels within the range classified as moderate or severe disease, and eight of them had resumed other treatments.

Currently, the most widely-used hemophilia A treatment is Roche's (ROG.S) antibody Hemlibra, which won U.S. approval in 2017. Given as a weekly injection, it works by bridging other factors in the blood to restore clotting. Unlike the gene therapies, which are approved only for adults, Hemlibra can be given to all ages, including infants.

Pfizer (PFE.N) on Monday said it is seeking U.S. approval for its experimental antibody marstacimab after showing positive Phase 3 data at ASH for both hemophilia A and B.

Pfizer and Roche are also developing gene therapies for hemophilia.

Other hemophilia researchers are looking to genetically modify a patient's own stem cells and re-introduce them, but without need for harsh transplantation conditioning regimens. Others are looking at ways to engineer a patient's B cells to produce blood factors; and to use gene editing to correct clotting factor production. "There is some incredible innovation," Recht said.

Reporting By Deena Beasley Editing by Caroline Humer and Bill Berkrot

Source: https://www.reuters.com/business/healthcare-pharmaceuticals/uptake-new-hemophilia-gene-therapies-slow-field-assesses-options-2023-12-15/

Octapharma USA: FDA Grants Expanded Approval to wilate® as the First VWF Concentrate for Prophylaxis in All Types of VWD

Dec 05, 2023

Routine Prophylaxis Now Recommended for Adults and Children Aged 6 and Older with Severe VWD

PARAMUS, N.J., Dec. 5, 2023 /PRNewswire/ -- Octapharma USA, Inc. announced the expansion of the U.S. Food and Drug Administration (FDA) approval for wilate®, von Willebrand Factor/Coagulation Factor VIII Complex (Human) Lyophilized Powder for Solution for Intravenous Injection. The approved label now includes routine prophylaxis aimed at reducing the frequency of bleeding episodes in adults and children aged 6 and older with any type of von Willebrand disease (VWD), the most prevalent bleeding disorder in the United States. Wilate® is the first von Willebrand factor (VWF) concentrate indicated for prophylactic treatment across all forms of VWD, marking a significant milestone in the field.

Octapharma USA announced the expansion of the U.S. Food and Drug Administration approval for wilate®, von Willebrand Factor/Coagulation Factor VIII Complex (Human) Lyophilized Powder for Solution for Intravenous Injection. The approved label now includes routine prophylaxis aimed at reducing the frequency of bleeding episodes in adults and children aged 6 and older with any type of von Willebrand disease (VWD), the most prevalent bleeding disorder in the United States.

"Long-term prophylaxis with VWF concentrate, as compared to on-demand treatment for bleeding, is recommended for patients with severe VWD," according to Shveta Gupta, MD, a specialist in pediatric hematology and oncology with The Haley Center for Children's Cancer and Blood Disorders at Orlando Health Arnold Palmer Hospital for Children. "The approval of wilate® for VWD prophylaxis is a welcome new treatment option that can be life-saving for many patients. Increased use of VWF prophylaxis in VWD patients may lead to improved patient care and a reduced burden of disease."

WIL-31 Study: A Breakthrough in VWD Treatment

The FDA approval is supported by Octapharma's WIL-31 study, a prospective, non-controlled, international, multicenter phase 3 trial that investigated the efficacy and safety of wilate® prophylaxis over 12 months in people aged 6 and older with severe VWD of any type.

All WIL-31 patients received on-demand treatment with wilate® during a previous six-month, prospective, observational study (WIL-29). Patients who experienced at least six bleeding episodes (BEs), excluding menstrual

bleeds, with at least two of these BEs treated with a VWF-containing product, were eligible to enter WIL-31. Patients in WIL-31 received wilate® prophylaxis two to three times per week at a dose of 20-40 IU/kg, for 12 months.

The clinical trial's primary purpose was to investigate whether prophylaxis with wilate® lowered the mean total annualized bleeding rate (ABR) by more than 50% compared to the six months of on-demand treatment. Secondary goals were to measure spontaneous ABR and treatment-emergent adverse events.1

Researchers reported an 84% reduction in the mean total ABR compared with on-demand treatment during the prior study. The median spontaneous ABR decreased by 95%. Importantly, no serious drug-related adverse events or thrombotic events were observed during the study.

Addressing the Prevalence of VWD in the U.S. Population

Von Willebrand disease affects up to 1% of the U.S. population, equating to about 3.3 million individuals. The disease, occurring equally among men and women, is often noticed more by women due to heavy or abnormal bleeding during menstrual periods and after childbirth. VWD includes three major types – Type 1, Type 2, and Type 3, the most severe form. The expanded wilate® label provides a treatment option for a significant patient population.2

"Wilate® prophylaxis for adults and children aged 6 and older with VWD promises to be life-changing for many patients," stated Octapharma USA President Flemming Nielsen. "Patients have been forced to live with far too many bleeding episodes while receiving on-demand treatment. Octapharma is pleased to offer patients a new therapy option that can greatly improve their quality of life."

Factor My Way: Empowering VWD Patients

In tandem with this approval, Octapharma USA extends its commitment to patient care through the Factor My Way patient support program. Tailored for those living with VWD and hemophilia A, this free membership program provides access to caregivers, educational programs, a resource library, and more. For additional information, please visit FactorMyWay.com.

About wilate®

Wilate®, von Willebrand Factor/Coagulation Factor VIII Complex (Human) Lyophilized Powder for Solution for Intravenous Injection is indicated in children and adults with von Willebrand disease for:

- On-demand treatment and control of bleeding episodes
- Perioperative management of bleeding
- Routine prophylaxis to reduce the frequency of bleeding episodes

Wilate® is indicated for routine prophylaxis in children 6 years of age and older and adults with von Willebrand disease.

Wilate® is indicated in adolescents and adults with hemophilia A for:

- Routine prophylaxis to reduce the frequency of bleeding episodes
- On-demand treatment and control of bleeding episodes

CONTRAINDICATIONS

Do not use in patients with known hypersensitivity reactions, including anaphylactic or severe systemic reaction, to human plasma-derived products, any ingredient in the formulation, or components of the container.

WARNINGS AND PRECAUTIONS

Anaphylaxis and severe hypersensitivity reactions are possible. Thromboembolic events may occur. Monitor plasma levels of FVIII activity. Development of neutralizing antibodies to FVIII and to VWF, especially in VWD type 3 patients, may occur. Wilate® is made from human plasma and carries the risk of transmitting infectious agents.

For complete prescribing information, please visit wilateusa.com/pi.

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2 – Centers for Disease Control and Prevention, What is von Willebrand Disease.

Source: https://www.biospace.com/article/releases/octapharma-usa-fda-grants-expanded-approval-to-wilate-as-the-first-vwf-concentrate-for-prophylaxis-in-all-types-of-vwd/

The year ahead

19 – 21 January, 2024

Youth (18-30) sailing

Auckland.

01 - 03 March, 2024

Teen and youth camp

MERC, Auckland.

26 - 28 July, 2024

Women's wellness weekend

Wairakei Resort, Taupō.

06 - 09 February, 2025

National family camp

Ngāruawāhia.

Visit www.haemophilia.org.nz for more information on bleeding disorders, HNZ News, and past issues of Bloodline.

Enjoy this issue ONLINE

Check your inbox for the online version today

Reading Bloodline online saves HNZ money, and is good for the environment.

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