CONTACT

Newsletter of Haemophillia Foundation WA Inc.

July 2022

Cover Picture: Photo taken from 2019 Australian Congress by Cheryl Ellis

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Nursing Staff: **Natalie Gamble-Williams** and **Stacey Hutchison**

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Medical Staff**: Dr Stephanie P'ng** and **Dr Dominic Pepperell**

Nursing Staff: **Sandra Lochore** and **Lara Olson**

Social Worker: **Helena Reynolds** (Mon and Thurs 9:00 am - 1:00 pm, contact for appointment) Ph: 6152 6527

ABDR Data Manager and Clinical Trial Coordinator: **Marina Goruppi**

Entrance to the Cancer Centre is on the outside of the building
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Level 1 Cancer Centre
Fiona Stanley Hospital
102-118 Murdoch Drive
Murdoch WA 6150

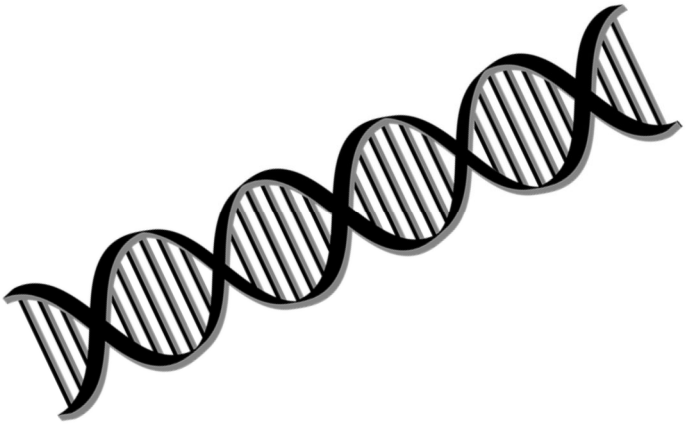
Postal Address (address all correspondence as Private and Confidential);
Haemophilia and Haemostasis Centre
Level 1 Cancer Centre
Fiona Stanley Hospital
Locked Bag 100, Palmyra DC WA 6961

President's Report

I hope this edition of the HFWA Newsletter - Contact finds everyone in good health and Covid free - It has been a frustrating period again for us at HFWA considering whether or not to go ahead with face-to-face Foundation activities/events. We were looking at recommencing Men's/Women's Breakfasts on 31 July but with the latest variants of Covid impacting significantly on the wider community with infections and hospitalizations, we have decided to postpone yet again the breakfasts. We hope to commence them as soon as it is safe and practical to do so , especially when they are indoor events.

The AGM of HFWA is to be held on 14 September 2022 at City West Lotteries House (see page 9 for details) it would be great to see as many of our community in attendance as possible, don't worry we won't be twisting people's arms to join the committee !!!

Please take time to read all of this Newsletter especially the article on Gene Therapy on pages 7 and 8, please note that all patients will qualify for gene therapy as up tp 50% of patients have antibodies to the vehicle used to insert the gene therapy these are called vectors - if you have antibodies you are unable to receive gene therapy, there are new vectors being investigated to allow more patients to access gene therapy



If you have any suggestions for future activities or education sessions that you believe that HFWA could facilitate please do not hesitate to contact us

Regards

Gavin Finkelstein

World Congress Plenary—By Cheryl Ellis

First presenter: Simon Fletcher (Lead Research Nurse, Oxford University Hospitals NHS Foundation Trust) "What patients with hemophilia tell us about living in the new paradigm of hemophilia treatment"

Simon refers to an article published in 2018 by M. Makris & C. Hermans about the current "Golden Age" for Treatments available for people with bleeding disorders; these being what the authors call 'disruptive treatments' that 'replace (for example, extended half-life products), rebalance (for example, novel products such as fituseran & emicizumab) & replicate (gene therapy)'.

Medical professionals DO NOT KNOW what it is like to have a bleeding disorder! This is quite a profound and eye-opening thing to realise as a person with a bleeding disorder. It illustrates the importance of completing quality of life surveys; with the results of these surveys, clinicians can understand what the "Golden Age" for treatments mean to people with bleeding disorders.

The surveys explore what improvements these treatments have made to patients' quality of life. Two of the surveys Simon spoke of were Emi & Me and Exigency. Emi & Me is a survey for patients being treated with Hemlibra (emicizumab), and Exigency is a gene therapy patient survey. In both surveys, patients reported that they felt decreases in pain levels, and perceived increases in freedom and control of their lives.

Second presenter: Ming Y. Lim (MBBChir, Associate Professor, University of Utah, Salt Lake City, USA) "Non-factor therapy in persons with acquired hemophilia A and von Willebrand disease"

Ming spoke of the effectiveness & high efficacy of emicizumab treatment in the treatment of acquired hemophilia A and type 3 (severe) von Willebrand Disease patients.

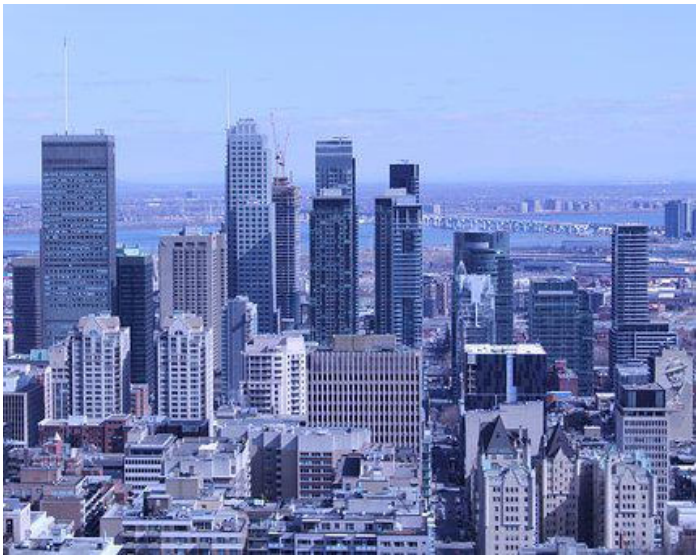
Acquired hemophilia A is a rare autoimmune disease that mostly affects the elderly and is a result of IgG antibodies against factor VIII (inhibitors). Acquired hemophilia A presents with severe bleeding characteristics, and often results in high morbidity and high mortality. Due to the low numbers of patients with acquired hemophilia A, studies are limited regarding treatment dosage levels.

Ming also spoke of a very small sample-size study of eight type 3 von Willebrand patients; three patients had detectible inhibitors and five patients had recurrent bleeds whilst on routine prophylaxis. The study patients were given standard loading and maintenance doses of emicizumab similar to hemophilia patients. All

the patients in the study achieved hemostasis after emicizumab treatment, and no adverse events were reported after twelve months of follow-up. Ming reiterated that this was a very small study, and that more data from clinical trials are required to reinforce and refine treatment for patients with these issues.

Third presenter: Sarah O'Brien (MD, MSc, Nationwide Children's Hospital, Columbus, Ohio, USA) "Recognising women with a bleeding disorder: A new diagnosis"

Sarah spoke of the new recommended ISTH nomenclature of female FVIII (hemophilia A) and FIX (hemophilia B) carriers, and the relevance of plasma factor levels and phenotypes (observable traits) when diagnosing females. Firstly, she looked at factor plasma levels of less than 0.40 IU/ml (40 units). Women and girls with these levels correlated with the categories for males with hemophilia A & B; mild - 0.05 to less than 0.40 IU/ml (5-less than 40 units), moderate – 0.01 – 0.05UI/ml (1-5 units), and severe – less than 0.01UI/ml (less than 1 unit). Secondly, Sarah



spoke of the use of clinical bleeding phenotypes to categorise females with plasma factor levels above 0.4UI/ml (40 units) as symptomatic (exhibited bleeding symptoms) or asymptomatic.

Sarah talked about several hurdles to diagnosis of women and girls with bleeding disorders. Some of these included the fact that many health care professionals still do not recognise that females CAN have a bleeding disorder; hemophilia does not just affect men. Women themselves sometimes do not realise that having a relative with a bleeding disorder means that they may also have low factor levels. The most commonly reported symptoms of carriers were from the studies examined by Sarah were heavy menses, oral cavity bleeding, post-partum hemorrhage and excessive bleeding after tooth extraction. Often, these symptoms are not recognised as bleeding disorder symptoms until after diagnosis. Education and advocacy, therefore, are important tools that enable women and girls to be diagnosed and receive effective treatment.

River Cruise

Save the date! HFWA will be hosting a river cruise on the 19th of November! This event will take place on a (hopefully) pleasant spring's day. Leave the kids at home, hop aboard the SS HFWA for a relaxing, enjoyable evening. We'll be floating down the Swan River, enjoying the twilight and having fun.

It'll be one of our first big social events for the year, after a hiatus due to the Covid pandemic. It's a great chance to reconnect to the community, to get involved and see other people. If you want to enjoy yourself, leave the kids at home, kick back and relax, it'll be a great opportunity! If you're interested, more information will be coming out in future issues of the newsletter, so keep your eyes out for that! It's sure to be a great opportunity, and one you'd hate to miss out on!

If you'd like to register your entry early on, feel free to contact us at office@hfwa.org and let us know you're keen!

Original photo of Swan River by Greg O'Beirne, available under CC-BY 2.5

First gene therapy to treat severe Haemophilia A recommended in Europe

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EMA has recommended granting a conditional marketing authorisation in the European Union (EU) for Roctavian (valoctocogene roxaparvovec) for the treatment of severe haemophilia A in adults who do not have factor VIII inhibitors (auto-antibodies produced by the immune system which make factor VIII medicines less effective) and no antibodies to adeno-associated virus serotype 5 (AAV5).

Patients with haemophilia A cannot produce factor VIII (an essential protein required for blood to clot and stop bleeding); they are more prone to bleeding and have prolonged bleeding, e.g. after injury or surgery. Haemophilia A is a rare debilitating disease affecting approximately 0.7 in 10,000 people in the EU. It is life long and may be life threatening when bleeding occurs in the brain, the spinal cord or the gut.

Medicines currently authorised for treating haemophilia A mostly contain factor VIII, to replace the missing protein. Available treatments require one or more injections per week or per month and are lifelong. Therefore, there is an unmet medical need for new therapeutic approaches that might free patients from frequent injections.

Roctavian is the first gene therapy to treat haemophilia A. The active substance in Roctavian, valoctocogene roxaparvovec, is based on a virus (adeno-associated virus or AAV) which has been modified to not cause disease in humans. The virus contains the gene for factor VIII; once given to a patient as a one-off infusion, it is expected to carry the factor-VIII gene into the liver cells, enabling them to produce the missing factor VIII. This helps the blood to clot more easily and prevents bleeding or reduces bleeding episodes. It is yet unknown how long the treatment effect from this single infusion will last in an individual patient. A sustained positive treatment effect of up to two years following a single infusion has been reported in approximately one hundred patients in the main study and up to five years in a few patients in a supportive trial conducted by the applicant. Longer-term follow-up tests may be required to verify a continued safe and effective response to the medicine.

EMA's recommendation is based on the results of a Phase 3 single-arm (main study), non-randomised study in 134 male patients with haemophilia A without a history of factor VIII inhibitor and without detectable pre-existing antibodies to AAV5. Two years after the administration, efficacy data showed that the therapy significantly increased factor VIII activity levels in the majority of patients. Bleeding rates were reduced by 85% and most patients (128) no longer needed factor VIII replacement therapy.

Hepatotoxicity (liver damage), a common side effect due to immune reaction induced by these AAV-based gene therapies and characterised so far by an increase in the levels of a liver enzyme called alanine aminotransferase (ALT), has been reported with Roctavian. The condition can be treated successfully with corticosteroids. Other common side effects include headache, joint pain and nausea.

Patients treated with Roctavian will be monitored for 15 years, to ensure the long-term efficacy and safety of this gene therapy.

Roctavian was supported through EMA's PRIority MEdicines (PRIME) scheme, which provides early and enhanced scientific and regulatory support to medicines that have a particular potential to address patients' unmet medical needs.

In its overall assessment of the available data, the Committee for Advanced Therapies (CAT), EMA's expert committee for cell- and gene-based medicines, found that the benefits of Roctavian outweighed the possible risks in patients with haemophilia A.

The CHMP, EMA's human medicines committee, agreed with the CAT's assessment and positive opinion, and recommended approval of this medicine.

The opinion adopted by the CHMP is an intermediary step on Roctavian's path to patient access. The opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role or use of this medicine in the context of the national health system of that country.

UPCOMING EVENTS

MEMBERSHIP RENEWAL: JULY

AGM: WEDNESDAY SEPTEMBER 14
AT CITY WEST LOTTERY HOUSE
FROM 7 PM

RIVER CRUISE: SATURDAY NOVEMBER 19

HFWA are selling quality shopping bags to support our state programs.

To order your bags and arrange collection

Jute Large Shopping Bag

Red Shopping Bag

Email your order for the shopping bags and / or nail polish directly to the HFWA office at office@hfwa.org with your name, address and phone number or phone 9420 7294 and we will advise when your order can be collected - or we will dispatch your order promptly by mail (postage can be arranged at extra cost)

For fast and secure payment, follow the secure Square credit card payment link below or simply scan the QR code to go straight to the Square checkout

https://checkout.square.site/merchant/06461WB19EDA8/checkout/R74ZARTGOWNVOO6YVONMBHMX

HFWA NAIL POLISH GIFT PACKS

Three bottle gift packs available.

Gift Packs $20

Gift packs contain Helen's Melons (red) and two col- ours of your choice.

Assorted Colours $10 each

Helen's Melons, Emerald Green (almost gone), White, Top Coat & Rose Gold

For fast and secure payment, please follow the secure Square credit card payment link below or simply scan the QR code to go straight to the Square checkout below for your Nail Polish purchase

https://checkout.square.site/merchant/06461WB19EDA8/checkout/V55VON3QLYYYU3PUWTEB65RA

Haemophilia Foundation of WA Inc.

APPLICATION FOR 2022/23 MEMBERSHIP

	New ☐ **Renewal** ☐	**TAX INVOICE** ABN 42 961 282 521 GST Registered
Title		
First Name		
Last Name		
Address		
Telephone		
Email		

To reduce the use of paper the HFWA Contact newsletter is delivered electronically.
Please tick ☐ if you would prefer to receive a printed copy via post.

PRIVACY:
HFWA membership automatically entitles you to Haemophilia Foundation Australia (HFA) membership. HFWA respects member's privacy. Your details will NOT be forwarded to other organisations, bodies, or persons without your permission. Please refer to the privacy statement on the HFWA website for details, http://www.hfwa.org/
Please tick ☐ if you do NOT want your details forwarded to HFA.

Please indicate:	
☐ Person with bleeding disorder	
☐ Grandparents	☐ Parent of Child
☐ Nurse	☐ Doctor
☐ Other	☐ Special Interest

Please return this membership form via email or to the address below:

☐ Individual ☐ Family (includes immediate family members) - **Membership $25.00 (GST inclusive)**

Extended family members need to take out their own membership.

Membership fee can be waived in special circumstances – Please contact the HFWA office on 9420 7294.

I would like to make a donation of:

☐ $25 ☐ $50 ☐ $100 or $ ***Donations over $2.00 are tax deductible***

☐ Direct Deposit	Acct Name: The Haemophilia Foundation of WA Inc. BSB: 086 488 Acct No: 035 233 031 Ref: *Please include your name e.g. John Smith*	
☐ Credit Card	Pay securely via the Square Payment link here or by scanning the QR code.	
☐ Cheque enclosed		

Haemophilia Foundation of WA Inc.

APPLICATION FOR 2022/2023 MEMBERSHIP
Family History

Please list all family members to be included in HFWA membership.

Name		**Date of Birth**		
Please indicate diagnosis details:				
☐ Haemophilia A		☐ Haemophilia B		
☐ von Willebrand Disorder		☐ Carrier		
☐ Other Factor Deficiency		☐ No Bleeding Disorder		
☐ Severe	☐ Moderate	☐ Mild	☐ vWD Type	
Relationship to Member:				

Name		**Date of Birth**		
Please indicate diagnosis details:				
☐ Haemophilia A		☐ Haemophilia B		
☐ von Willebrand Disorder		☐ Carrier		
☐ Other Factor Deficiency		☐ No Bleeding Disorder		
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☐ Other Factor Deficiency		☐ No Bleeding Disorder		
☐ Severe	☐ Moderate	☐ Mild	☐ vWD Type	
Relationship to Member:				

Signature:			Date:	
OFFICE USE ONLY	RECEIVED	REC. NO.	ENTERED	HFA