19th Australian Conference

on haemophilia, VWD & rare bleeding disorders

Challenging the Status Quo

~ 10-12 October 2019 ~

Abstracts & Handbook

HAEMOPHILIA FOUNDATION AUSTRALIA

WELCOME

Welcome to the 19th Australian Conference on haemophilia, VWD & rare bleeding disorders. We hope you enjoy the conference and find it a stimulating and informative meeting. We encourage you to participate actively to add to the richness of this exciting conference.

We sincerely thank the Program Committee for bringing what we hope will be a very exciting and informative meeting to you. The hard work and professional commitment is greatly appreciated.

Gavin Finkelstein
President
Haemophilia Foundation Australia

Dr Liane Khoo
Chair
Conference Program Committee

Sharon Caris
Executive Director
Haemophilia Foundation Australia

Program Committee

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Alison Morris	Australian and NZ Physiotherapy Haemophilia Group
Suzanne O'Callaghan	Policy Research and Education Manager, Haemophilia Foundation Australia
Loretta Riley	Australia/NZ Haemophilia Social Workers' & Counsellors' Group
Robyn Shoemark	Australian Haemophilia Nurses' Group

GENERAL INFORMATION

Conference Organisers

Haemophilia Foundation Australia
7 Dene Ave, Malvern East VIC 3145
P: 03 9885 7800 F: 03 9885 1800
E: hfaust@haemophilia.org.au W: www.haemophilia.org.au

Venue

Novotel Manly, Sydney
55 North Steyne, Manly

Disclaimer

All information in the Conference Program and Abstracts is correct at the time of printing. The organisers may alter the Conference Program in the event of unforeseen circumstances. Some abstracts may not have been available at the time of print. Daily program changes will be notified during the Conference.

Mobile Phones

As a courtesy to delegates and speakers, please switch off or set your mobile phones to silent during all sessions. If you need to take a call please do not answer your mobile until you have left the room.

Name Tags

Entrance to the Exhibition area and Conference sessions will be limited to name tag holders only. If you misplace your name tag, please advise HFA staff at the Registration and Information Desk.

Internet

Wireless internet is available for all conference delegates
Password: H1

Registration and Information Desk

All enquiries should be directed to the Registration and Information Desk located in the main exhibition area and will be open at the times listed below:

Thursday 10 October	17:00-19:30
Friday 11 October	07:30-17:00
Saturday 12 October	07:30-15:35

Hospitals for haemophilia treatment

There will be no treatment room at the conference venue. Delegates must make their own plans for medical services and treatment. All Delegates should plan well in advance to bring sufficient supplies of haemophilia treatment products to meet treatment requirements.

ROYAL PRINCE ALFRED HOSPITAL, HAEMOPHILIA CENTRE
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T 02 9515 7013
Emergency 02 9515 6111

THE CHILDREN'S HOSPITAL AT WESTMEAD
Cnr Hawkesbury Rd & Hainsworth St Westmead NSW 2145
T 02 9845 1138
Emergency 02 9845 0000 & page haematologist on call

SYDNEY CHILDREN'S HOSPITAL, CENTRE FOR CHILDREN'S CANCER & BLOOD DISORDERS
High St Randwick NSW 2031
Doctor 02 9382 1690
Nurse 02 9382 1240
After Hours 02 9382 1111 & ask for haematologist on call

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Darcy Rd, Wentworthville NSW 2145
T 02 8890 5187

Childcare

Childcare is not available at the Conference and children are not permitted in Conference sessions.

SOCIAL PROGRAM

Thursday 10 October

Welcome & Exhibition Opening
18:30-19:30
Exhibition
Come to see the exhibition and meet people before the conference. Free for all registered delegates.

Youth Meet and Greet
19:30 til late
Lobby Lounge and Bar
Youth are invited to meet others and connect before the conference program starts. Free to all registered youth delegates.

Friday 11 October

Remembrance Service
18:00-18:45
Terrace
The Remembrance Service is a time to remember friends and family, and the people we have cared for. The service is non-religious and everyone is welcome.

Conference Dinner
19:00 til 21:30
Ballroom
Come and join your fellow delegates for an informal dinner and to celebrate Haemophilia Foundation Australia's 40th birthday. The dinner will be free seating.

Dinner tickets must have been purchased in advance -
no tickets will be available during the conference or on the night.

Saturday 12 October

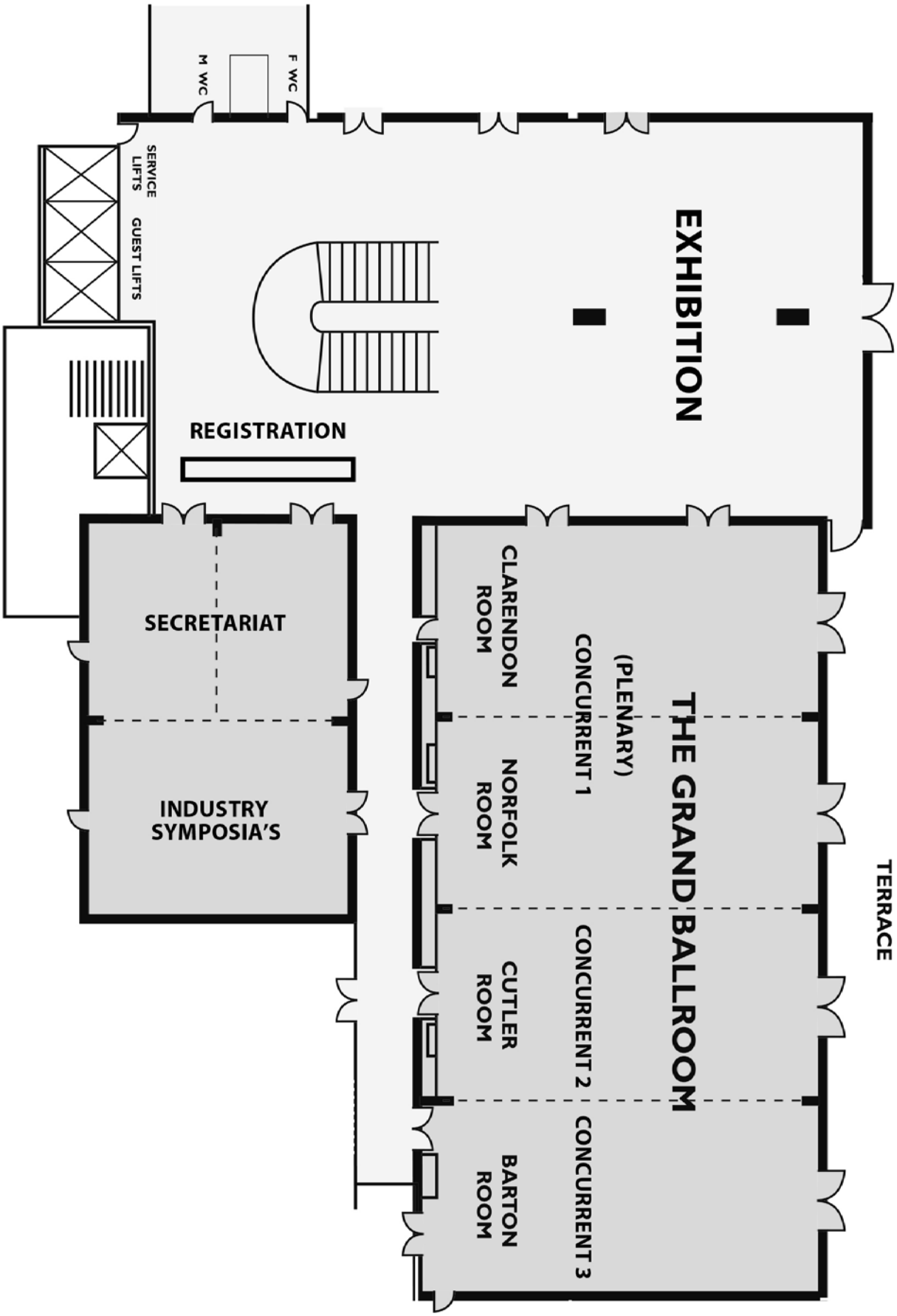
Men's Breakfast ~ Everything you wanted to know but were afraid to ask
07:00 – 08:20
Room: Ballroom Terrace

Women's Breakfast ~ Extraordinary women in leadership: some of the inspiring women I have met
07:00 – 08:20
Room: Barton Room

Tickets to both the Men's and Women's breakfast must have been pre-purchased –
no tickets are available during the conference.

CONFERENCE VENUE MAP

– LEVEL 1



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POSTER ABSTRACTS

The poster display is in the main foyer area. All posters will be displayed for the duration of the conference. Poster authors will be available at their poster the following times for discussion and questions.

Friday 11 October
10.20-10.30

	Author	Co-Authors	Title
1	Ross Paterson		Order and Chaos
2	Sharon Lee	Claire Arcé, Brian Branchford, Daniel Hart, Sarah Hendry, Maria Kelleher, Michelle Kim, Robert Ledniczky, Mimi Lee, Sharon Lee, Matthew Minshall, Claude Negrier, Steve Prince, Michelle Rice, Robert Sidonio*	Optimizing language for effective communication of gene therapy concepts: A qualitative study
3	Lucy Mayo	Dr Emily Ward, Mrs Auburn McIntyre, Dr Helen Banwell	The ankle and haemophilia: a new measurement tool
4	Loretta Riley and Graham Norton		Connecting the Community through Art
5	Abi Polus		The role of the physiotherapist in acquired haemophilia: A review of the literature and clinical review
6	Jane Portnoy	LÝ THI HẢO	A Tale of two Social Workers

Saturday 12 October
10.20-10.30

	Author	Co-Authors	Title
7	Prof Laurie L Brown	H.A. La1; J. Li1; M. Brunner[2], M Snoke[2], A.M. Kerr[2]	Societal Costs of Haemophilia A in Australia
8	Alex Connolly	Beryl Zeissink	Case study of a haemophilic pseudo tumour in a patient with severe haemophilia A with a high titre inhibitor
9	Susan Webzell		Current Nursing Workloads and Implications for the Future within Haemophilia Treatment Centres in Australia.
10	Anne Jackson	Jane Nyaketcho, John Nelson Opio, Sara Carlino, Dr Manika Pal, Dr Malaika Perchard	Emicizumab – Reducing the impact on children with inhibitors and improving outcomes
11	Dr Simon Fifer, CaPPRe Annette M Kerr, Roche Products, Pty. Limited Australia Claire Parken, Roche Products, Pty. Limited Australia Dr Kim Hamrosi, CaPPRe* Miss Samantha Eid, CaPPRe		Understanding Treatment Preferences for Haemophilia A in Australia – Patient and Caregivers of Children with Haemophilia A
12	Preetha Jayaram	Suzanne O'Callaghan	Getting Older Project needs assessment
13	Dr Simon Brown & Dr Jonathan Holzmann	Hayley Coulsen, Wendy Poulsen, Joanna McCosker, Moana Harlen	Emicizumab therapy leading to resolution of a massive iliopsoas haematoma and allowing removal of port-a-cath in an adolescent with severe haemophilia a, chronic high titre FVIII inhibitor and previous pulmonary embolism.
14	Jaime Chase	Dr Janis Chamberlain & Dr Emma Prowse	It takes a Village- The Implementation of a Multidisciplinary Clinic for Children and Adolescents with Bleeding Disorders.

POSTER 1

Name: Ross Paterson
Institution/Organisation: Haemophilia Foundation of New Zealand
Title: Order and Chaos

1. Outline and description of topic/content of presentation/project
In this presentation I share a relational component of my work undertaken with a member of the bleeding disorders community I am an outreach worker for.

Building relationships of trust and mutual respect are critical in developing partnership as a framework to work together. In this instance a casual conversation during one of our meetings revealed a mutual interest in hill walking. This may seem a small insight but finding common ground is the beginning stage of opening the door to working and learning together. We began walking in the Port Hills of Christchurch (a beautiful but at times unforgiving landscape) and encouraged other Foundation's members in the Christchurch to join us with the notion of promoting resilience and wellbeing amongst our community.
The population in the greater Christchurch area have been impacted by "chaos" and those with enduring health needs carry a higher burden of stress. I have a small role in facilitating their return to "order" (in this instance by supporting their health, decreasing stress and improving social connections). This presentation outlines the challenges relating to "the chaos" and describes strategies that contribute to bringing about change with the goal of returning to "order".

2. Conclusions/outcomes
I will explore emerging themes and describe how they have impacted on my practice.

Discussion and Strategies for moving ahead:
I outline the theories that underpin this work and how they inform the approach to partnership. I reflect with input from the group what is working, what could work better and where they see this approach can be improved.

POSTER 2

Name: Sharon Lee
Co-authors: Claire Arcé, Brian Branchford, Daniel Hart, Sarah Hendry, Maria Kelleher, Michelle Kim, Robert Ledniczky, Mimi Lee, Sharon Lee, Matthew Minshall, Claude Negrier, Steve Prince, Michelle Rice, Robert Sidonio*
Institution/Organisation: BioMarin Pharmaceutical Inc.
Title: Optimizing language for effective communication of gene therapy concepts: A qualitative study

For communities of persons living with hemophilia and other genetic conditions, gene therapy could represent a paradigm shift in treatment strategies. As therapeutic modalities become increasingly complex, there is a critical need for all stakeholders (patients, physicians, patient advocates, nurses, caregivers, reimbursement agencies, drug developers, and regulators) to communicate with a lexicon that is intelligible, accurate, and consistent. In doing so, expectations can be more carefully managed and potential risks, benefits, and limitations can be better understood.

In recognition of this need, a first-ever study of gene therapy lexicon was conducted. Here, we report findings that identify a recommended language set for effectively communicating information about adeno-associated virus (AAV)-based gene therapy for hemophilia, between and among stakeholders.

Structured screener interviews were used to identify a total of 84 suitable participants representing five individual countries (US, UK, Spain, France, Germany, Italy) and audiences (hematologists, nurses, caregivers, patients, and patient advocates). Then, a series of in-depth interviews, face-to-face focus groups, advisory board meetings, and online group interviews were held to collect, refine, and test language and image concepts. Sessions were conducted in local languages with detailed discussion guides.

Across multiple topics, preferred words, phrases, and pictorial representations were developed and agreed upon through an iterative and adaptive process. Undesirable, disagreeable, or confusing language was identified. Preferences were largely consistent across audiences and countries; however, where differences existed, country-specific recommendations were made.

Study results show that the hemophilia community has preferences around consistent lexicon used to describe hemophilia and its therapeutic approaches. Further, outcomes suggest that the use of preferred language can increase understanding and comfort during discussions of novel and complex therapeutic modalities such as gene therapy. In summary, this study suggests that consistent use of community-informed lexicon can minimize miscommunication and facilitate informed decision-making regarding potential future treatment opportunities. **Authorship alphabetical*

POSTER 3

Name: Miss Lucy Mayo
Co-authors: Dr Emily Ward, Mrs Auburn McIntyre, Dr Helen Banwell
Institution/Organisation: The University of South Australia
Title: The ankle and haemophilia: a new measurement tool

Background
The ankle joint is the most commonly affected in individuals with haemophilia. The immediate stages of a bleed include blood accumulation within the joint space, reducing the range of motion (ROM) available. The ankle joint is fundamental for lower limb function and adequate ROM is important for essential daily tasks, such as walking. This study aimed to test if parents are able to use a new tool to measure ankle ROM in a repeatable and consistent way and get the same results as a physiotherapist.

Methods
To determine the ability of a physiotherapist, physiotherapist in training and parent to measure ankle ROM consistently on two different occasions and compared to one another. A clinical inclinometer, iPhone and tape measure were used to measure ankle ROM in a weight bearing lunge (WBL) position. 13 participants from the Women's and Children's Hospital, South Australia, were positioned and measured by each rater in randomised order.

Results
The study determined that the physiotherapist, physiotherapist in training and parent were able to measure consistently on two separate occasions with each device (all ICC values > 0.80). Additionally, measurements with both an iPhone and tape measure by a parent and physiotherapist were very similar when compared (ICC>0.80). The tape measure showed the highest consistency for all raters individually on repeated occasions and when compared to each another (ICC>0.95).

Conclusion
Due to the irreversible nature of joint damage caused by haemophilia, early identification and factor administration is imperative to minimise joint damage and future haemophilic arthritis. The WBL and iPhone provide a reliable method for parents to monitor ankle joint status, increasing the ability of families to self-manage their child's haemophilia. Due to recent advances in factor replacement therapy, this tool may provide an indication for administration and limit joint damage.

POSTER 4

Author/s: Loretta Riley and Graham Norton
Institution/Organisation: Queensland Haemophilia Centre
Title: Connecting the Community through Art

World Haemophilia Day 2019, saw a joint collaboration between Haemophilia Foundation Queensland and the Queensland Haemophilia Centre to promote inherited bleeding disorders to the wider community while simultaneously offering support to the affected community. An Art Exhibition was held throughout April in the public viewing area of the RBWH. The exhibition featured photographs, abstract art work, graphic art/cartooning, watercolours, acrylic, woodwork and ceramics from members of the inherited bleeding disorders community. In true art gallery style, an "opening' event was held to coincide with World Haemophilia Day (challenging the status quo and holding it mid-way through the exhibition) on the 17th April, with staff of the Royal Brisbane and Women's Hospital, Haemophilia Foundation Queensland and members of the inherited bleeding disorders community and their families attending.

Connecting with the wider community in terms of art was an effective health promotion activity. Information about inherited bleeding disorders could be shared in a subtle way, through posters, quotes and artist's bios. Anecdotal feedback from RBWH staff who visited the exhibition was that the most
effective information came from the artist bios and comments from the inherited bleeding disorder community, with this information shining a light into the experience of living with an inherited bleeding disorder.

Enabling members of the bleeding disorders community to share their work had the intended outcome of utilising creative strategies to manage pain, stress and utilise meaningful activities to enhance wellbeing. It also showed the wider community that people are more than their diagnosis or role in the community.
Connections were an unexpected outcome, with the "opening event" creating an opportunity for active members in the community to engage with other families of someone with a bleeding disorder to gain enhanced support and encouragement

to attend other peer support activities. Unexpectedly, given that many of the artists themselves were diagnosed with a range of inherited bleeding disorders, it also provided an opportunity for an informal discussion around other diagnoses, inviting compassion and understanding within the community itself.

POSTER 5

Name: Abi Polus
Institution/Organisation: The Alfred
Title: The role of the physiotherapist in acquired haemophilia: A review of the literature and clinical review

Acquired haemophilia is becoming a more frequent presentation in hospitals due to better identification and management. The importance of physiotherapy in the management of bleeding disorders is well documented as is suggested from the time of diagnosis and onwards (Srivastava et al., 2012). Clinical experience physiotherapy is an integral part of management in acquired haemophilia due to the pathology and its consequences.

Acquired haemophilia is an extremely rare bleeding disorder (1.5/million diagnoses per year) that is caused by autoantibody production against a coagulation factor, usually factor VIII, which inhibits the procoagulant function. This condition is not hereditary and development is spontaneous. It has been associated with other autoimmune conditions, underlying malignancy, pregnancy, or drug induced, although it is estimated that 50% remains idiopathic. It is characterized clinically by bleeding predominantly into the subcutaneous, muscle, mucosal membrane and soft tissues.

The average age of onset has been documented as 74-78, with a small peak in women in childbearing years. It has been suggested that many people within this cohort, who develop acquired haemophilia, will already have an alternate or underlying disorder, for which they may require care. Management of acquired haemophilia includes homeostatic pharmacological control of the bleeding and management of the inhibitor by steroids and cytotoxic agents; these medications are well documented in potentially causing iatrogenic issues, including sepsis, unstable blood sugar, confusion, skin reactions and mood changes which may affect mobility and musculoskeletal function. Physiotherapy is indicated in the management of musculoskeletal dysfunction to allow return to pre-morbid function.

A literature search was undertaken to identify if any previous literature discussed the role of physiotherapy in acquired haemophilia. Two papers discussed physiotherapy in acquired haemophilia; one case study (Goto et al, 2016) and a one line comment stating the patient recovered ‘following intensive physiotherapy’ (Forsythe et al., 2012).

We undertook an observational study of our records of patients with acquired haemophilia at our adult state centre for bleeding disorders, which receives tertiary referrals from hospitals throughout the state and provides specialist care. An in depth audit of notes allowed the summary of physiotherapy input. Over 50% of patients admitted received physiotherapy input. This ranged from one off assessment to long term input.

Education, advice, reassurance, gait aid provision and progression to independence, as well as rehabilitation of affected muscles and joints comprised the musculoskeletal management of a large proportion of these patients' management. The importance of physiotherapy in the management of patients with acquired haemophilia should not be underestimated and further study should be undertaken to evaluate the role.

POSTER 6

Name: Jane Portnoy
Co-authors: LÝ THI HẢO
Institution/Organisation: Alfred Health and NIHBT
Title: A Tale of two Social Workers

Background
Jane is a social worker in Melbourne from Alfred Health and was asked to join a delegation to Vietnam, Hao is the head social worker at NIHBT in Hanoi. When we met we began a dialogue about the similarities and differences of our work. There are specifics of social work in the area of bleeding disorders. These Haemophilia services have worked together for over 20 years. This was the first time that a Social Worker from Australia was included. Social Work with the Haemophilia patients at the NIHBT started in 2015. There are many shared issues and some significant differences in the work that social workers do.

Methods
Participation in an international training program at NIHBT in Vietnam.

Aims
Engage in a discussion, begun in person and continued via email about the work we do and how we can work together.

Discussion
What are the challenges for haemophilia social work in Vietnam? Are they relatable to our own?

Results
Despite obvious differences, there were many commonalities in the role of the social worker. With huge numbers of patients, difficulty getting treatment, and severe financial hardship, the Vietnamese social workers also consider "education, personal career, finance, family, treatment support, personal problem defining."

There was an instant synergy with the Vietnamese and Australian social workers. The work has commenced, now we have an opportunity for ongoing communication, research and sharing of resources and experiences.

POSTER 7

Name: Professor Laurie L Brown[1]
Co-authors: H.A. La[1]; J. Li[1]; M. Brunner[2], M Snoke[2], A.M. Kerr[2]
Institution/Organisation:
[1] NATSEM, Institute for Governance and Policy Analysis, University of Canberra, ACT, Australia.
[2] Roche Products Pty Limited, Sydney, Australia
Title: Societal Costs of Haemophilia A in Australia

1. Outline and description of topic/content of presentation/project
BACKGROUND: In Australia, haemophilia A (HMA) occurs in over 2,200 adults and children, with varied disease severity. Between 60% and 80% of people with HMA report at least one bleeding episode annually; most commonly affecting the joints, muscles and/or soft tissue. People with HMA require lifetime intravenous treatment with recombinant or plasma derived Factor VIII (FVIII) or bypassing agents (BPAs); disease severity and FVIII inhibitor status determining the treatment regimes. AIM: To identify and quantify direct and indirect societal and economic costs of HMA in Australia under current treatment practices. METHODS: Using a cost of illness approach, direct costs were defined as the cost of 'resources' expended on people with HMA, including health care and related support or non-medical expenses (out-of-pocket costs for individuals, their family or carers) and government support payments. Indirect costs are defined as value of lost productivity by people with HMA or their carers, potentially impacting on superannuation accumulation, reduced savings and personal or family assets, as well as reduced income tax revenue for Government.

2. Conclusions/outcomes
RESULTS: Treatment costs (FVIII blood products and BPAs) were estimated to be $84.5 million or an average of $91,282 per patient per year, contributing to 87% of direct costs and 76% of total costs. Indirect costs contributed to $14.3 million or an annual cost per patient of $15,425, and 12.9% of total costs. Lost productivity accounted for 56% of all indirect costs. Overall, the total cost of moderate and severe HMA in Australia in 2018 was estimated to be $111.1 million; or an average annual cost of $119,956 per patient. CONCLUSIONS Consistent with other reported studies, the cost of blood products (including BPAs) dominated expenditure, representing 76.1% of total annual costs. The second highest cost, lost productivity, highlights the need for new treatments that improve quality of life and workforce participation.

POSTER 8

Name: Alex Connolly
Co-authors: Beryl Zeissink
Institution/Organisation: Queensland Haemophilia Centre
Title: Case study of a haemophilic pseudo tumour in a patient with severe haemophilia A with a high titre inhibitor

Introduction:
This is a case report of our experience in the management of this rare condition in a 48- year old man recently immigrated to Australia who presents with a chest wall pseudotumour and until presentation had an unknown high titre inhibitor to factor VIII and limited understanding of Haemophilia.

Aims:
Engage patient and his family with the Haemophilia Treatment Centre (HTC). Educate patient and family on bleeding symptoms, reconstitution and administration of rFVIII. Commence patient on immune tolerisation therapy (ITT) to attempt to eradicate inhibitor.

Methods:
Regular clinic visits were scheduled with an interpreter present. Education provided through interpreter. Peripheral Inserted Central Catheter (PICC) inserted due to patient's poor venous access. Patient's niece was educated on how to reconstitute rFVIII and administer rFVIII via PICC line. Education provided around PICC maintenance and signs of infection as well as signs of bleeding.

Results:
Patient's niece established a constructive and positive relationship with the HTC as the family spokesperson. Home therapy established initially and then taken over by niece. ITT ceased after four months and PICC removed due to PICC line sepsis. Unable to place new PICC due to thoracic outlet syndrome. Bleeds managed with conservative treatment and recombinant factor VIIa (Novoseven®).

Conclusions:
This case report highlights the ongoing clinical and therapeutic challenges associated with a patient from a non-English speaking background and limited understanding of Haemophilia, inhibitors and bleed management.

POSTER 9

Name: Susan Webzell
Institution/Organisation: Hollywood Hospital
Title: Current Nursing Workloads and Implications for the Future within Haemophilia Treatment Centres in Australia.

Background:
Patients with haemophilia and other bleeding disorders require specialist management. In Australia, Haemophilia Treatment Centres (HTC's) have a multidisciplinary team (MDT) to manage these patients. In a changing environment of care, these centres are constantly evolving to encompass new treatments and technologies often with little or no increase in resources.

Aims:
To determine, through comparison of historical data – if HTC workloads and nursing responsibilities have changed/increased?

Analysis of current resources, nursing responsibilities and expectations to identify workload implications.

Methods:
The Australian Haemophilia Nursing Group (AHNG) were invited to examine their centres by completing a questionnaire and followed up with more focused questioning. Details collected included types of patients, patient numbers, working hours for nursing, medical, allied health and data administration/ clerical alongside reviewing nursing roles. Adult and paediatric centres were looked at separately.

The current data was compared with historical data to see comparison of resources and changes in workload.

Roles and responsibilities of Haemophilia nurses were examined.

Results:
There is significant variation between centres, their resources and their patient numbers.

Most have significant input in surgeries with many HTCs coordinating care of patients being treated outside the HTC hospitals. Clinical trials occur in most centres with or without input from specialist clinical trials nurses. It was also identified there was difficulty finding time for nursing led research across the centres.
The data suggests increased workloads with minimal changed resources to accommodate these increases. The current roles seen in the data collected are in keeping with those identified in a survey on global roles.

Conclusion:
There have been many changes and increases in the demands to the role of the Haemophilia Nurse.

Strategies to overcome the identified implications are required at both a national and international level.

POSTER 10

Name: Anne Jackson
Co-authors: Jane Nyaketcho, John Nelson Opio, Sara Carlino, Dr Manika Pal, Dr Malaika Perchard
Institution/Organisation: Women's & Children's Health Network
Title: Emicizumab – Reducing the impact on children with inhibitors and improving outcomes

Introduction:
This paper presents the process to best practice outcomes and partners with parents of a 4 year old boy to describe the journey of diagnosis and treatment of severe haemophilia A with an inhibitor. We report the impact on them and their child's life, modifying his environment and activities, the frequent hospital visits and the dramatic change to quality of life after emicizumab commencement.

Discussion:
Being diagnosed with severe haemophilia is highly stressful for families. The diagnosis of an inhibitor increases the impact on the daily life for the child. Eradicating inhibitors involves daily dosing of Factor 8 which becomes the role of parents in the home. Episodes of painful bleeding can occur requiring assessment and treatment in hospital. This becomes an additional burden on their lives and reduces the possibility of normal activities.

Recommended treatment for this patient involved commencing a tolerisation program with standard half-life recombinant FVIII and treatment with Novoseven for bleeding.

It became evident that this was not a satisfactory approach as he continued to bleed. Over a period of 104 days from date of inhibitor he had 36 bleed free days. He had 67 presentations to hospital in this period. In view of this the HTC advocated for access to emicizumab and successfully gained approval and funding via Women's & Children's Health Network and SA Department of Health. This was commenced 13th February 2019. The benefits of emicizumab were immediate with a reduction in presentations to hospital to 17 out of 106 days. Patient has been bleed free and has returned to normal activities.

Conclusion:
Accessing emicizumab had significant positive results with no bleeding, reduced visits to hospital and improved quality of life. This demonstrates that advocating for new treatments for improved outcomes is a priority for HTC's.

POSTER 11

Details of author/s: Dr Simon Fifer, CaPPRe, Annette M Kerr, Roche Products, Pty. Limited Australia
Claire Parken, Roche Products, Pty. Limited Australia, Dr Kim Hamrosi, CaPPRe*, Miss Samantha Eid, CaPPRe
Title: Understanding Treatment Preferences for Haemophilia A in Australia – Patient and Caregivers of Children with Haemophilia A

1. Outline and description of topic/content of presentation/project
BACKGROUND: Hemophilia A (HMA) is a bleeding disorder characterised by a deficiency in factor VIII (FVIII). Intravenous FVIII replacement has been the standard of care in severe HMA.
AIM: To assess preferences for treatment characteristics of prophylactic HMA therapies in people with HMA (with and without current FVIII inhibitors) and their caregivers.

METHODS: An online survey with a discrete choice experiment (DCE) was used to quantify the treatment preferences of people with HMA (with and without current FVIII inhibitors) and their caregivers. The DCE consisted of two treatment alternatives labelled by mode of administration (subcutaneous injection, intravenous infusion). The treatment attributes focused on the clinical benefits, pharmaceutical benefits and associated risks and were selected based on previous research and expert opinion. The option to stay on their current treatment was also included. A Latent Class Model (LCM) was estimated from the DCE data.

2. Conclusions/outcomes
RESULTS: The LCM (n= 51) identified two segments based on treatment preferences. Segment 1 (larger segment) preferred treatments administered by a subcutaneous injection over intravenous infusion and a treatment with little to no risk of developing inhibitors and a low number of bleeds per year. Additionally, those with current FVIII inhibitors preferred treatments that reduced their number of bleeds per year and with a low risk of mild side effects. Segment 2 (smaller segment) had a similar focus on the main treatment features to segment 1 but also favored treatments that could be used as both a rescue and prophylaxis, had low risk of experiencing any side effects, and had low risk of an injection site reaction.

CONCLUSION: There exists heterogeneity between treatment preferences for people with HMA (with and without FVIII inhibitors) and their caregivers. Results from this study could be used to guide informed decisions around the value of existing treatments to people with HMA and their caregivers.

POSTER 12

Name: Preetha Jayaram
Co-authors: Suzanne O'Callaghan
Institution/Organisation: Haemophilia Foundation Australia
Title: Getting Older Project needs assessment

Getting Older is a Haemophilia Foundation Australia priority project. It aims to identify, understand and respond to the range of needs people with bleeding disorders may have as they are getting older and help find appropriate solutions for them and their partner/family/carers now and into the future.

The project will consider what 'future proofing' would involve for this group: anticipating what may occur in the future and developing methods to minimize any negative effects of future events and maintaining health and wellbeing into the future.

Over the last several decades, advances in treatment and care for people with bleeding disorders have seen significant improvements in quality of life. Many people with bleeding disorders are living longer lives than ever before. Many live with significant mobility issues and complications due to joint and muscle damage from repeated bleeds in their earlier years, when there were treatment shortages However, they are also beginning to experience the complications associated with ageing in the general community, and earlier in life compared to the rest of the population. What are the implications of this for them and their partners and family in relation to self-management, comprehensive care, and planning for the challenges of life in the future as they grow older? How can they best maintain their independence and age positively?

This poster will present the key findings from the HFA Getting Older needs assessment. The findings will be based on consultation with community members with bleeding disorders and their partners/family, state and territory foundations, medical specialists, haemophilia nurses, psychosocial workers and physiotherapists, and will explore current issues and how to 'future proof' as people grow older. The poster will also outline some solutions proposed, including online options for community members to inform themselves and connect with each other, as well as other possibilities.

POSTER 13

Name: Dr Simon Brown[1] and Dr Jonathan Holzmann[1]
Co-authors: Hayley Coulsen[2], Wendy Poulsen[2], Joanna McCosker[3], Moana Harlen[3]
Institution/Organisation: Children's Health Queensland
Title: Emicizumab therapy leading to resolution of a massive iliopsoas haematoma and allowing removal of port-a-cath in an adolescent with severe haemophilia a, chronic high titre FVIII inhibitor and previous pulmonary embolism

Introduction: Inhibitor formation in children with severe haemophilia A remains the most significant complication of FVIII therapy. Treatment of bleeding episodes in individuals with a FVIII inhibitor utilises by-passing agents (BPA) such as rFVIIa and APCCs. Recent studies have reported prevention of bleeds in patients with inhibitors using a novel monoclonal antibody, Emicizumab. We report a case where Emicizumab therapy lead to the resolution of a massive retroperitoneal haematoma originating from the iliopsoas muscle, and allowed removal of a port-a-cath.

Results: A 15-year-old male, who had failed immune tolerization, and had a chronic high titre FVIII inhibitor (maximum 3328 BU/mL) presented in haemodynamic shock with a massive retroperitoneal bleed (estimated 2L, nadir Hb 64 g/L) despite daily BPA prophylaxis. He had a history of recurrent musculoskeletal bleeds (ABR >30), chronic arthropathy (HJHS 25) with subsequent mobility issues, regularly missed school due to bleeds, and previously suffered a pulmonary embolus (PE) during BPA therapy. The acute bleed was controlled with BPA therapy. A port-a-cath was re-inserted (complicated by a large wound haematoma) to facilitate intensified BPA therapy for the iliopsoas bleed. An application for compassionate use Emicizumab was lodged a month before the bleed. Emicizumab therapy (3mg/kg weekly sc injections for 4 weeks and 1.5mg/kg weekly injections thereafter) was approved and commenced 14 weeks after the initial bleed. On Emicizumab the TEG normalised, the haematoma (pre-Emicizumab size 6 x 4 x 5 cm) fully resolved over 19 months, the ABR was 0, school attendance increased and the port-a-cath was removed without BPA therapy. No complications or adverse events were encountered on Emicizumab.

Discussion/Conclusion: Emicizumab is a novel monoclonal antibody that bridges FIXa with FX, activating FX and thereby bypassing FVIII. This case demonstrates the safe and effective use of Emicizumab for over 20 months in an individual with a prior history of PE complicating his severe haemophilia A with a chronic high titre FVIII inhibitor.

POSTER 14

Name: Jaime Chase
Co-authors: Dr Janis Chamberlain and Dr Emma Prowse
Institution/Organisation: Children's Cancer and Haematology Service- John Hunter Children's Hospital
Title: It takes a Village- The Implementation of a Multidisciplinary Clinic for Children and Adolescents with Bleeding Disorders.

Traditionally, the care of children and adolescents with a bleeding disorder at John Hunter Children's Hospital, Children's Cancer and Haematology Service was comprised of six-monthly reviews with the child/adolescents Consultant. The Consultant would refer the

children and their families to other members of the healthcare team as required: referrals could take time in being processed and the family would need to attend hospital for additional appointments. School aged children and adolescents were required to have additional time away from schooling and friendship groups to attend these appointments. Adherence to appointments was identified as being suboptimal.

In 2018 the John Hunter Children's Hospital Bleeding Disorders clinic was launched. This clinic comprises of review by key members of the multi-disciplinary team (MDT) within one clinic appointment. The child/ adolescent and their families are reviewed by the Consultant, Nurse Specialist, Social Work, Psychology, Physiotherapist and Child Life Therapy. Appointments are 2 hours in total duration and take place in the afternoon to enable children and adolescents to attend morning schooling commitments.
Within these appointments the child/ adolescent is assessed for medical, education, social, physical and psychological needs by the use of screening tools, brief clinical assessment, one on one education and support.

It is anticipated that annual review within the MDT clinic setting will lead to improved health outcomes of children and adolescents with bleeding disorders and lead to the increased utilisation of available healthcare services. We attribute this to increased opportunities to develop relationships with all members of the healthcare team. The MDT clinic allows for early intervention across all disciplines and the identification of concerns before they become problematic.

In the year since the clinic's inception, the MDT team has had the following results:

- 95% of joint scores completed
- 80% increase in school visits and education completed
- 50 % increase in the utilisation of medical alert identification
- 90% of children/adolescents and parents/guardians screened for psychological distress and appropriate referrals completed as required. (allowing for identification of psychosocial concerns, so that these can be addressed by treating clinicians)

Bleeding disorders are lifelong disorders that effect both the individual and family. Annual MDT clinics enable the child/adolescent and family to be both personally and holistically assessed. We believe that empowering families leads to increased interaction and utilisation of healthcare services.

CONFERENCE PROGRAM

THURSDAY 10 OCTOBER 2019

0830- 1700	**Annual meetings of AHCDO, AHNG, ANZHSWCG, ANZPHG, DMG** (Health professionals' group members only)
1830-1930	**Welcome and Exhibition Opening** – open to all registered conference attendees
1930-2230	**Youth VIP Meet & Mingl**e – for registered youth only

FRIDAY 11 OCTOBER 2019

0840	**Official Conference Welcome**
	Gavin Finkelstein, President HFA Dr Teresa Anderson, Chief Executive RPAH
0845-1015	**Plenary 1: Improving outcomes - what has been achieved in the treatment of bleeding disorders?** **Chair: Dr Liane Khoo** **Room: Claredon/Norfolk**
	Introduction and current treatments ~ Dr Liane Khoo Personal Experience ~ Dr Simon McRae and John Optimising patient care through PK analysis (WAPPS) ~ Prof Alfonso Iorio Q&A
1015-1045	**MORNING TEA**

1045-1215	**Concurrent 1** **Addressing the challenges of inhibitors** **Room: Claredon/ Norfolk**	**Concurrent 2** **Getting older** **Room: Cutler**	**Concurrent 3** **Looking after children who have a bleeding disorder** **Room: Barton**
	Chair: Megan Walsh	**Chair: Frankie Mullen**	**Chair: Robyn Shoemark**
	A clinical overview ~ Dr Huyen Tran Discussion and debate on immune tolerance induction therapy ~ Dr Chris Barnes and Dr Julie Curtin Personal journey ~ Andrew	Emerging clinical issues ~ TBC Patient voices: quotes from needs assessment ~ Preetha Jayaram Growing older with your HTC ~ Penny McCarthy Maintaining independence and keeping up with exercise ~ Greig Blamey Innovative projects in ageing ~ Marcia Fearn Q&A	Encouraging independence and overcoming barriers to self-infusion ~ Robyn Shoemark Psychosocial issues for parents and families with a child with a chronic illness ~ Dr Emma Prowse Transition and personal perspective - Anne Jackson & Patrick When can I play again? Q&A on physiotherapy issues, joint health and sport ~ Alison Morris and Johanna Newsom
1215-1325	**LUNCH**		

	Concurrent 1 **360° on clinical trials** **Room: Claredon/ Norfolk**	**Concurrent 2** **Reproduction and family planning** **Room: Cutler**	**Concurrent 3** **A healthy life for all ages** **Room: Barton**
	Chair: Dr Tina Carter	**Chair: Jaime Chase**	**Chair: Beryl Zeissink**
	The role of your doctor in a clinical trial ~ Dr Simon Brown The role of nurses in trials ~ Stephen Mathews A family's experience of a clinical trial for children ~ Alicia An individual's experience of a clinical trial ~ Hamish Patient reported outcomes to improve the value of clinical trials in haemophilia (PROBE) ~ Dr Alfonso Iorio Q&A	A joint presentation on reproduction and family planning • Kristi Jones ~ Geneticist • Lucy Kevin ~ Genetics Counsellor Personal Stories • Kara • TBC	Managing a healthy diet and weight ~ Jules Aitken Exercising safely at all ages ~ Greig Blamey Personal experience of managing haemophilia challenges and keeping fit and well ~ Tim Q&A
1455-1535	**AFTERNOON TEA**		

1535-1700	**Plenary 2** **Room: Claredon/Norfolk**
	Chair: Dan Credazzi
	Dr Happy (Dr Tim Sharpe) ~ Challenging the status quo
1800	**Remembrance Service** **Room: Terrace**
1900 til late	**Conference Dinner** **Tickets are $50 and must be pre-paid** **Room: Ballroom**

SATURDAY 12 OCTOBER 2019

0700 - 0820	**Men's Breakfast ~ Everything you wanted to know but were afraid to ask** *Tickets are $30 and must be pre-paid* **Room: Terrace**
	Chair ~ Claude Damiani Panel: Greig Blamey, Tim, Zev, Dr Nalini Pati
0700 - 0820	**Women's Breakfast ~ Extraordinary women in leadership: some of the inspiring women I have met** *Tickets are $30 and must be pre-paid* **Room: Barton**
	Chair ~ Sharon Caris Marie Ann Fernandes (World Federation of Hemophilia Regional Program Manager for Asia & Western Pacific) will share her experiences as a development worker in different parts of the world
0830-1000	**Plenary 3 Musculoskeletal challenges: joint care and treatment** **Room: Claredon/Norfolk**
	Chair: Abi Polus
	The importance of upper limb care throughout life ~ Greig Blamey The Utility of Ultrasound in the evaluation and management of Haemophilic Arthritis ~ Dr Rob Russo Surgical options ~ Dr Mark Horsley
1000-1045	**MORNING TEA**

1045-1215	**Concurrent 1** **Gene therapy de-mystified** **Room: Claredon/ Norfolk**	**Concurrent 2** **From girls to women** **Room: Cutler**	**MORNING TEA** **Concurrent 3** **Self-advocacy** **Room: Barton**
	Chair: Dr Susan Russell	**Chair: Susan Dalkie**	**Chair: Loretta Riley**
	The basic science of blood clotting and how this interacts with new therapies ~ Dr Yvonne Brennan Through the Looking Glass into the World of Gene Therapy and Non-Factor Therapies ~ Dr Julie Curtin	Personal story: self-management, diaries, what a parent wants to know ~ Shauna Female Factors - issues for different life stages and how to handle them ~ Dr Jenny Curnow The Gynaecologist and Patients with Bleeding Disorders ~ Dr Kim Mathews Carriers: What do parents, young girls and women need to know? ~ Joanna McCosker Q&A	An interactive workshop: successful self-advocacy; identifying relevant skills and resources; barriers and overcoming them. Session to be facilitated by Loretta Riley with Dr Liane Khoo, Nicoletta Crollini and Jane Portnoy
1215-1325	**LUNCH**		

1325-1455	**Concurrent 1** **Von Willebrand disease and rare bleeding disorders** **Room: Claredon/ Norfolk**	**Concurrent 2** **Youth** **Room: Cutler**	**Concurrent 3** **What is comprehensive Care today?** **Room: Barton**
	Chair: Dr Ritam Prasad	**Chair: Moana Harlen**	**Chair: Anne Jackson**
	Current issues and VWD - best practice and future treatment ~ Dr Ritam Prasad Developing an effective treatment plan ~ Alex Connolly Personal experience of living with VWD and being a parent of a child with VWD ~ Susie The rare bleeding disorders ~ Dr Heather Tapp Personal experiences of living with a rare factor deficiency ~ Belinda Q&A	What's the risk? Panel discussion of scenarios, with audience input (Q&A style) - personal decision-making around visiting HTC for a bleed review; gym/sport-related injury; travel, insurance; disclosing – in sport, in relationships; work, challenges of becoming an adult. Panel: • Young people with bleeding disorders ~ Alan • Older person with haemophilia and parent ~ Paul and Shane • Health professionals ~ • Greig Blamey, Physiotherapist • Jane Portnoy, Psychosocial worker • Steve Matthews, Haemophilia Nurse	Oral Health and Dental Management of Patients with Bleeding Disorders ~ Dr Suma Sumakura Navigating Health Care and Other Systems ~ Loretta Riley Queensland Haemophilia Centre's experience of Telehealth – engaging with the rural, remote and regional inherited bleeding disorders community ~ Loretta Riley, Amy Finlayson and Dr Jane Mason
1455-1535	**AFTERNOON TEA**		

1535-1700	**Plenary 4** **New opportunities or is the status quo good enough?** **Room: Claredon/Norfolk**
	Chair: Dr Simon McRae
	What patients want to achieve from treatment – impact of new treatment to a person's life. From a parent's point of view ~ Claude Damiani New Therapies for haemophilia: can we achieve new goals? ~ Prof Alfonso Iorio Nurse – nursing into the future ~ Robyn Shoemark Expert on evaluation of new therapies, payment models, reducing barriers to access ~ Michael Stone Panel Discussion

ABSTRACTS

FRIDAY 11 OCTOBER 2019
0845-1015
Plenary 1: Improving outcomes - what has been achieved in the treatment of bleeding disorders?
Chair: Dr Liane Khoo

Introduction and current treatments ~ Dr Liane Khoo

Personal Treatment Experience ~ Dr Simon McRae and John

Optimising patient care through PK analysis (WAPPS) ~ Prof Alfonso Iorio
The main contribution of adopting individual pharmacokinetic (PK) profiling to hemophilia management has been and is bringing the patient front and center. PK was traditionally used to compare concentrates, but its application to individual patients can help matching patients' life goals with a treatment plan built on their own individual response to factors. Increase the dose or the frequency of treatment in response to bleeding or suggesting performing or not intense physical activity on a given day can be based on science and not educated guess. Population PK, and the adoption of modern IT technology, enables us to generate interactive graphs representing the individual PK profile over a week of treatment, building on an individual PK study involving 2-3 samples taken during regular prophylaxis infusions. Several barriers have been abolished, such as complex PK studies, difficult PK calculation and interpretation, translating PK into a treatment recommendation, communicate the plan to the patient, engage patients in their own optimal care. When using a service like the Web-Accessible-Population-Pharmacokinetic-System for hemophilia (WAPPS-Hemo, www.wapps-hemo.org), PK studies can be performed without wash-out, and each individual study is checked out and validate by a PK expert. The results are reported as time to critical plasma levels (5, 2 and 1%). A clinical module help building an individual interactive graph representing the plasma factor level for a specific combination of treatment dose and interval, also providing peak, trough, and weekly time above 15%, 3% and 1% and usage to compare different treatment regimens. This curve can be transferred to a mobile application intended for patients (myWAPPS), which will show the current and future plasma factor level after a recorded plasma concentrate infusion. Availability and adoption of this technology can help achieving more effective and feasible treatment in hemophilia.

FRIDAY 11 OCTOBER 2019
1045-1215
Concurrent 1
Addressing the challenges of inhibitors
Chair: Megan Walsh

A clinical overview ~ Dr Huyen Tran
Discussion and debate on immune tolerance induction therapy ~ Dr Chris Barnes and Dr Julie Curtin
Personal journey ~ Andrew

FRIDAY 11 OCTOBER 2019
1045-1215
Concurrent 2
Getting older
Chair: Frankie Mullen

Emerging clinical issues ~ TBC

Patient voices: quotes from needs assessment ~ Preetha Jayaram
What challenges do people with bleeding disorders face in their day to day life as they are getting older? This presentation offers examples of those experiences lived or felt by the bleeding disorder community members who have participated in HFA's Getting Older Needs Assessment Project. It reflects the aspirations, concerns, uncertainties and needs of those affected.

Growing older with your HTC ~ Penny McCarthy
This presentation will touch on some of the complexities of care for the ageing population of people with inherited bleeding disorders and the role of the Haemophilia centre providing ongoing care

Maintaining independence and keeping up with exercise ~ Greig Blamey
Medical management of haemophilia has seen rapid advancement in recent years with the advent of extended half-life products and novel therapies, but the full impact of these therapies is less likely to be realized by older adults, and the ability to provide greater strength, stamina, fitness and therefore the opportunity for maximised quality of life remains the domain of exercise and physical activity. This presentation will focus on how incorporation of exercise into a lifelong routine promotes independence with reference to issues faced by people with haemophilia as they age. The concepts of ageing and enhanced musculoskeletal fitness are both undergoing revolutionary change at the present time with respect to historical perceptions of what can be achieved by people with a bleeding disorder – a paradigm shift that stands to benefit all people with haemophilia across the lifespan.

Innovative projects in ageing ~ Marcia Fearn
Due to improvements in medical care, the life expectancy of people living with haemophilia and other bleeding disorders is increasing. Getting older can affect a person's health and wellbeing, and older people are more likely than younger people to have multiple long-term health conditions.
This increased life expectancy may bring with it many other issues or concerns that the bleeding disorder community have not experienced in the past. They now not only need to consider their bleeding disorder but also need to consider other medical comorbidities that can come with ageing, such as cardiovascular disease, dementia, arthritis, depression, loneliness or social isolation and an increased falls risk.

This presentation will discuss how some of these comorbidities, such as an increased falls risk, depression and dementia, can impact on the ageing bleeding disorder community. More importantly we will discuss some of the strategies that have been used to reduce the impact these issues can have on the ageing population, including, but not limited to, effective falls prevention programs, befriending programs for peer support, volunteering, social programs such as Men's Sheds, and some pain management strategies.

FRIDAY 11 OCTOBER 2019
1045-1215
Concurrent 3
Looking after children who have a bleeding disorder
Chair: Robyn Shoemark

Encouraging independence and overcoming barriers to self-infusion ~ Robyn Shoemark

Psychosocial issues for parents and families with a child with a chronic illness ~ Dr Emma Prowse
Children with chronic health conditions, such as bleeding disorders, are faced with a unique set of challenges, in addition to the typical challenges associated with growing up. These include feeling different from their peers and health related anxiety. Many parents also experience additional stress trying to protect their child from harm, which is complicated by a chronic health condition and can lead to further conflict in adolescence. How can we help children to thrive despite their chronic health condition? Transition and personal perspective - Anne Jackson & Patrick

When can I play again? Q&A on physiotherapy issues, joint health and sport ~ Alison Morris and Johanna Newsom
Even with better availability of prophylaxis, children with bleeding disorders still develop joint and muscle bleeds which require not just immediate treatment, but also ongoing rehabilitation. This rehabilitation phase is often very slow and steady, and the most common question the physiotherapist hears is “When Can I Play Again?” This presentation will combine lessons learnt from other areas, including elite sports rehabilitation and animal studies with current research in haemophilia care, which are used together to guide our answers.

FRIDAY 11 OCTOBER 2019
1325-1455
Concurrent 1
360° on clinical trials
Chair: Dr Tina Carter

The role of your doctor in a clinical trial ~ Dr Simon Brown
Participating in clinical trials can be exciting, daunting, petrifying and rewarding for all involved. Ultimately the responsibility for the conduct of the clinical trial at a site rests on the shoulders of the Principal Investigator (PI), which is often the senior medic in the team. Navigating clinical trials for a Medic is a significant task that consumes many additional hours of work. What are the responsibilities, what consumes so much time and what is it like to be a Medic involved in a clinical trial? Hopefully we can travel through this journey together and successfully navigate the obstacles.

The role of nurses in trials ~ Stephen Mathews
This presentation will explore the various potential roles and functions of the nurse involved in a clinical trial.

A family's experience of a clinical trial for children ~ Alicia

An individual's experience of a clinical trial ~ Hamish

Patient reported outcomes to improve the value of clinical trials in haemophilia (PROBE) ~ Dr Alfonso Iorio
The goal of treatment is the cure of a disease. For hemophilia it is correcting the bleeding defect an make the life experience of a person with hemophilia the same as for one without hemophilia. How can we select the measures appropriate to assess whether the goal is achieved with the treatment under study? First, we have to understand the chain of events leading to the impact of the disease on a person's life. For example, joint bleeds lead to arthropathy, which limits function and causes pain; in turn, these decrease participation in life activities; the fear of bleeding leads to risk avoidance, sedentarism, decreased socialization, lower social achievement; treatment have side effects, and cause disability and decreased life expectancy. Second, we have to learn how to precisely perform the measure and when. Bleeds can be counted every year, pain over any time interval. Joint function or work achievements require much longer observation periods. Outcomes not directly perceived by patients (called "surrogate", like plasma factor activity) can be measured when they have shown to be associated with patient important outcomes (hard outcome, like death or quality of life). Some outcomes can be objective measures (like a joint score), others can only be reported by the patient (like pain); others are in between (like bleeds). Third, we have to be able to measure the difference between patients receiving or not treatment, or subject with and without hemophilia, or change over time. The PROBE instrument has been developed and validated as a comprehensive set of outcomes. Built by patients for patients, PROBE is available in over 50 languages and generates patients and non-hemophilia controls data. The goal of the PROBE initiative is to provide a complete set of measures in hemophilia, also supporting their interpretation.

FRIDAY 11 OCTOBER 2019
1325-1455
Concurrent 2
Reproduction and family planning
Chair: Jaime Chase

A joint presentation on reproduction and family planning

- **Kristi Jones ~ Geneticist**
- **Lucy Kevin ~ Genetics Counsellor**

Personal Stories

- **Kara**
- **TBC**

FRIDAY 11 OCTOBER 2019
1325-1455
Concurrent 3
A healthy life for all ages
Chair: Beryl Zeissink

Managing a healthy diet and weight ~ Jules Aitken
Approximately one-third of the European and North American haemophilia population are overweight or obese – this includes children and adults. Being overweight or obese can cause a significant disease burden on joints already vulnerable to inflammation, and haemophilic arthropathy. Excess weight is associated with a decreased range in motion of joints, accelerated loss of joint mobility, overall reduced mobility and increased chronic pain. It also increases the risk of long-term chronic diseases such as heart disease, diabetes, and cancer.

Overweight children experience breathing difficulties, increased risk fractures, high blood pressure, exhibit early markers of cardiovascular disease and insulin resistance. Being overweight as a child is associated with a higher chance of disability in adulthood and premature death.

Maintaining a healthy weight reduces the burden on joints and long-term chronic health conditions. Obesity is preventable, but losing weight and maintaining a healthy weight for many is not as simple as exercise more and eat less. Physiological, genetic, environment, socio-economic status and psychology all play a part in energy balance.

This presentation aims to provide an understanding of how hormones affect hunger, satiety and fat storage, how to make smarter food choices and weight loss treatments available to assist with weight loss and maintenance.

Exercising safely at all ages ~ Greig Blamey
The haemophilia community is well versed in the notion of prophylaxis as a pillar of effective treatment with respect to factor replacement therapies. Similarly, the role of exercise has become well understood as having both protective as well as recuperative roles for people with bleeding disorders of all ages. Combining the two concepts into one and expanding the traditional definition of prophylaxis is a core concept of this presentation. Beyond the roles of treatment modalities, the roles of treatment team members has expanded over the years to include practitioners with unique skills that people with haemophilia can leverage into results that improve their quality of life. As options for physical activity for people with haemophilia expand, so must the opportunities that treatment teams make available to their patients. This presentation will detail a unique program from one Canadian clinic merging the expertise of a Physiotherapist and a Certified Personal Trainer with cooperation and operational funding from the Canadian Hemophilia Society

Personal experience of managing haemophilia challenges and keeping fit and well ~ Tim

FRIDAY 11 OCTOBER 2019
1535-1700
Plenary 2
Dr Happy (Dr Tim Sharpe) ~ Challenging the status quo
Chair: Dan Credazzi

Dr Tim Sharp is Australia's very own ' Dr Happy', at the forefront of the positive psychology movement as founder of The Happiness Institute.

A sought after public speaker, best-selling author of multiple books and regular on Australian and International TV, Dr Happy is widely regarded as a leader in mental health and the authority on all things happiness in Australia.

With three degrees in psychology (including a Ph.D.) and an impressive record as an academic, clinician and coach, Dr Happy is a passionate professional with a wealth of experience both on the field and in the media. Actively involved in research, education and practice, he is the founder and CHO (Chief Happiness Officer) of The Happiness Institute, Australia's first organisation devoted solely to enhancing happiness in individuals, families and organisations.

SATURDAY 12 OCTOBER 2019
0830-1000
Plenary 3
Musculoskeletal challenges: joint care and treatment
Chair: Abi Polus

The importance of upper limb care throughout life ~ Greig Blamey
This presentation will explore the consequences to overall function that present in the face of upper extremity bleeding and musculoskeletal deterioration. Core elements of fitness that are not always attributed to the upper extremity are central to Physiotherapy assessment and treatment and will be examined in the context of how their compromise impacts on activities of daily living. The presentation will examine how skilled Physiotherapy practice can mitigate the impacts of upper extremity bleeding and promote the maintenance of function throughout the lifespan.

The Utility of Ultrasound in the evaluation and management of Haemophilic Arthritis ~ Dr Rob Russo
The use of imaging has long had a role in the management of joint disease in those with Haemophilia. Traditionally X-rays were used to assess the severity of joint disease; However, disease is only recognised once sufficient damage has already occurred. MRI imaging is considered the Gold Standard; However, practical issues render this modality as problematic (particularly in the paediatric population). As such Ultrasound has emerged as the ideal approach to imaging given its accessibility, reproducibility, and the information it provides. This session will explore the utility of Ultrasound, using the HEAD-US approach, in regards to the diagnosis of joint disease as well as its contribution towards clinical decision making in relation to treatment.

Surgical options ~ Dr Mark Horsley

SATURDAY 12 OCTOBER 2019
1045-1215
Concurrent 1
Gene therapy de-mystified
Chair: Dr Susan Russell

The basic science of blood clotting and how this interacts with new therapies ~ Dr Yvonne Brennan
Have you ever wondered how blood clots or what the blood tests mean? This presentation aims to teach non-clinicians how blood clots, explain the blood tests that are done to measure blood clotting, and how the new haemophilia drugs affect blood test results.

There are 3 key players to blood clotting: platelets, von Willebrand factor and coagulation factors. Abnormalities in any of these key players can result in bleeding disorders. The laboratory can measure the amount of platelets and how well they function. Similarly, the amount and function of von Willebrand factor can be measured. Individual coagulation factor levels can be measured (e.g. factor VIII level, factor IX level), while the screening tests for coagulation factors are called PT and APTT.

Through the Looking Glass into the World of Gene Therapy and Non-Factor Therapies ~ Dr Julie Curtin
Since its first inception gene therapy has been an attractive option that may one day provide a cure for Haemophilia. Over the last 10 years great strides have been made in the development of gene therapy, such that now it is time to really look at Gene Therapy and what it has to offer. At the same time a number of other non-factor products have also been developed that are also changing the landscape in the management of haemophilia. These technologies are offering an unprecedented array of potential therapies for patients with haemophilia. In this session we will look through the looking glass and see what this new world of novel therapies really has to offer.

SATURDAY 12 OCTOBER 2019
1045-1215
Concurrent 2
From girls to women
Chair: Susan Dalkie

Personal story: self-management, diaries, what a parent wants to know ~ Shauna

Female Factors - issues for different life stages and how to handle them ~ Dr Jenny Curnow
If knowledge is power then understanding your bleeding disorder and how it impacts your experiences during different life stages can help women to optimise self-care. Menorrhagia is common but not always recognised. Various management strategies exist and can help prevent complications like endometriosis and iron deficiency anaemia. Many clotting factors increase during pregnancy and bleeding may be reduced but knowing what to expect during delivery, understanding your anaesthetic options and how to manage vaginal bleeding after delivery can help reduce concerns about having a family.

The Gynaecologist and Patients with Bleeding Disorders ~ Dr Kim Mathews
This presentation will explore the role of a gynaecologist in childhood, puberty and beyond, both in general, and specifically as it relates to those with bleeding disorders. Management of menstrual issues, transition to adulthood and later fertility issues will be covered.

Carriers: What do parents, young girls and women need to know? ~ Joanna McCosker
An exploration of physical and psychosocial issues for young girls and women who are carriers. An age appropriate developmental framework for young girls and women who are obligate or potential carriers. What education and care do they need at each stage, what should parents and caregivers think about and what do they need to know?

SATURDAY 12 OCTOBER 2019
1045-1215
Concurrent 3
Self-advocacy
Chair: Loretta Riley

An interactive workshop: successful self-advocacy; identifying relevant skills and resources; barriers and overcoming them.

Session to be facilitated by Loretta Riley with Dr Liane Khoo, Nicoletta Crollini and Jane Portnoy

SATURDAY 12 OCTOBER 2019
1325-1455
Concurrent 1
Von Willebrand disease and rare bleeding disorders
Chair: Dr Ritam Prasad

Current issues and VWD - best practice and future treatment ~ Dr Ritam Prasad

Developing an effective treatment plan ~ Alex Connolly

Personal experience of living with VWD and being a parent of a child with VWD ~ Susie

The rare bleeding disorders ~ Dr Heather Tapp
Rare bleeding disorders represent a challenge to the clinician because of the considerable differences in bleeding phenotype, the absence of clinical trials to guide management and the frequent lack of a specific replacement therapy. An overview of the rare bleeding disorders will be provided in this session.

Personal experiences of living with a rare factor deficiency ~ Belinda

SATURDAY 12 OCTOBER 2019
1325-1455
Concurrent 2
Youth
Chair: Moana Harlen

What's the risk?

Panel discussion of scenarios, with audience input (Q&A style) - personal decision-making around visiting HTC for a bleed review; gym/sport-related injury; travel, insurance; disclosing – in sport, in relationships; work, challenges of becoming an adult.

Panel:
Young people with bleeding disorders

- **Alan**

Older person with haemophilia and parent

- **Paul**
- **Shane**

Health professionals ~

- **Greig Blamey, Physiotherapist**
- **Jane Portnoy, Psychosocial worker**
- **Steve Matthews, Haemophilia Nurse**

SATURDAY 12 OCTOBER 2019
1325-1455
Concurrent 3
What is comprehensive care today?
Chair: Anne Jackson

Oral Health and Dental Management of Patients with Bleeding Disorders ~ Dr Suma Sumakura

Patients with congenital bleeding disorders have an increased risk of significant bleeding following invasive dental procedures. This presentation will cover the importance of maintaining oral health in patients with haemophilia, von Willebrand Disease and other rare bleeding disorders. There will be particular emphasis on the specific treatment planning considerations and modifications to the delivery of dental treatment in this patient cohort, with reference to patient cases that have accessed the dental service at Westmead.

Navigating Health Care and Other Systems ~ Loretta Riley

Navigating health care and other systems can be challenging, especially when you have a rare health condition. To add to the challenge, there are different processes and eligibility criteria for all the different organisations we may have contact with in our daily lives. Potentially there are also different specialists and allied health professionals you may need to interact with in order to look after your health and wellbeing. Sometimes it can be confusing where to turn to. During this presentation, examples of the NDIS, MyAged Care, Centrelink and health services will be described to assist with navigation of these support services

Queensland Haemophilia Centre's experience of telehealth – engaging with the rural, remote and regional inherited bleeding disorders community ~ Loretta Riley, Amy Finlayson and Dr Jane Mason

Queensland is the second largest Australian State with the most decentralized population. However, most specialist services are in South East Queensland including the Queensland Haemophilia Centre. As an addition to Outreach visits, both the Queensland Children's Hospital team and the Royal Brisbane and Women's Hospital team have expanded their services to include telehealth clinics in order to improve access to appointments with the Centres. Both teams will share their experiences; including the challenges and benefits of telehealth.

SATURDAY 12 OCTOBER 2019
1535-1700
Plenary 4
New opportunities or is the status quo good enough?
Chair: Dr Simon McRae

What patients want to achieve from treatment – impact of new treatment to a person's life. From a parent's point of view ~ Claude Damiani

New therapies for hemophilia: can we achieve new goals? ~ Prof Alfonso Iorio
Historically hemophilia patients have received a suboptimal treatment, as the property and cost of treatment did only permit to target minimally protective levels (e.g. 1%). Emerging treatments hold the promise of higher achievements. Non-factor replacement therapies as a whole a target plasma factor activity concentration in the mild hemophilia range (15-40%). Gene therapy, EHL factor IX and VWF-decoupled Factor VIII could enable factor activity levels constantly in the normal range (>40%). Measuring the incremental value of these innovative treatments emerges as the new challenge. A first problem is that traditional outcomes may still play a role but require a new interpretative framework. Spontaneous and traumatic bleeds need to be distinguished and correlated with the level of physical activity. Quality of life, pain interference, capacity of performing activities of daily living gain importance. A second problem is that the path from discovery to market access involves not only showing efficacy and safety (clinical stage and regulatory approval), but also comparative appraisal of effectiveness (Health Technology Assessment) and assessment of the value generated by treatment (Health Policy/Reimbursement decisions). Different outcomes are needed at different stages, but the traditional sequential approach may delay the availability of treatment by many years. Multi-stakeholder projects like CoreHEM or HemoValue have identified the minimum core of outcomes ensuring a complete appraisal of emerging technologies and are currently incorporated into ongoing clinical trials. A common set of outcomes will also enhance the capacity to compare different treatments, or assess their efficacy, safety and value across classes of treatments, again shortening the time needed to build a large enough body of evidence to allow decision making. The 6 outcomes identified by CoreHEM for gene therapy are frequency of bleeding, plasma factor level, duration of effect, chronic pain, cost, phycological impact.

Nurse – nursing into the future ~ Robyn Shoemark

Expert on evaluation of new therapies, payment models, reducing barriers to access ~ Michael Stone

NOTES

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