pnhsaa | PNH Support Association Australia Inc

Supporting Australians with Paroxysmal Nocturnal Haemoglobinuria

RARE BUT REAL

NEWSLETTER ISSUE №5 MAR 2015

Event Spotlight

National Conference and Annual General Meeting 2014

PNHSAA Conference Attendees

The second National Member's Conference was held on Saturday 30th August 2014 at RACV Club's picturesque resort in Torquay and it was bigger and better than the first conference held back in 2012 with over 65 people in attendance.

The day commenced with an opening address by the PNHSAA president Matt Dean who recapped on the year that was and celebrated 5 years since the incorporation of PNHSAA by summarising the key achievements over that time.

The first session was a presentation by Professor Harshal Nandurkar, Director of Haematology, St. Vincent's Hospital Melbourne, who provided an entertaining and informative overview of PNH and current research and treatment.

The second session was an overview and report of the PNH Registry, an important and vital resource for PNH patients and medical professionals. This session was presented by Ms Jean Young, Associate Director Medical Affairs, Alexion Pharmaceuticals.

The third session presented by Mr Michael Brown, Clinical Nurse, Royal Melbourne Hospital covered practical issues in clinical management including Soliris safety in compliance, risk of meningococcal and implications of infection, portacaths and travelling with PNH. The presentation was followed by an educational question and answer session with lots of audience participation.

The last session of the day was an open forum that provided all members the opportunity to provide feedback and suggestions to the committee members.

All points raised will be addressed by the committee in the coming 12 months.

Following the formal sessions of the day was the 2014 Annual General Meeting (AGM) and conference dinner.

The AGM provided the opportunity for members to be voted into the Committee. We are pleased to announce that the Committee Members voted in for 2015 are:

President: Mathew Dean
Treasurer: Michael Patterson
Secretary: Jenny Sturrock
Committee Representatives: Grace Dean, Chhean Khoun & Eve Mulder

Eve is a new addition to the Committee and we look forward to having her on board with new ideas and fresh perspective. The Committee looks forward to another successful year in 2015.

The Committee would like to thank all members who attended the event and your positive feedback. We would also like to extend our thanks to Prof. Harshal Nandurkar, Mr Michael Brown, Ms Jean Young and all the staff at RACV Club Torquay Resort for their assistance in making the conference a success.

More photos of the event can be seen on the website http://www.pnhsaa.org.au

Prof. Harshal Nandurkar

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A few words

A message from the President

I would like to wish all our members and supporters a very Happy New Year and I hope you all enjoyed the holiday period, as its now March I'm sure the fad diets are in full swing and the gym gear is back on.

2014 was a big year for the PNHSAA and our members with another successful National Conference which produced record attendance, and I'm sure this was a testament to the informative nature of the sessions and not just the location within Victoria's famous beachside town of Torquay.

In terms of the year to come we are excited here at the PNHSAA for a push to increase the awareness of PNH through social media and to continue our involvement and participation with Rare Voices Australia. In addition the PNHSAA has been invited to speak at a medical conference in the coming month to advise and educate others on what it takes and what it means to setup a patient support group. This will provide further exposure for PNHSAA.

Besides the interesting articles and updates for members, the newsletters this year will focus on informative pieces for our members with input from various medical experts who support the PNHSAA. I highly encourage members if you have ideas for articles that you wish to see included in the newsletter or any other ideas for support services to please get in contact with the PNHSAA.

As always a big thanks to the Committee for their efforts last year and thank you to all those members who continue to financially support us as without you there would be no PNHSAA.

Matt Dean, President

EVENT SPOTLIGHT

Rare Disease Event: Canberra ACT

In late 2014 two of our members, Wendy and Michael Patterson represented the PNHSAA in Canberra as part of a Rare Diseases Day event run by Rare Voices Australia.

Rare Voices Australia is a national, not for profit organisation established in 2012 with a vision to be 'the unified voice for ALL Australians living with a rare disease'. PNHSAA is endeavouring to support RVA in its quest to influence governments around Australia on a range of common issues that affect people with rare diseases. These include:

- Giving a unified voice to an estimated 1.2 million Australians living with a rare disease
- Rare Diseases need to be listed as a key health priority
- Advocate for a National Rare Disease Plan
- Co-ordination of services allowing for improved diagnosis, care and access to quality services in the area of rare disease.

The function attended by Wendy and Michael was held at Parliament House, Canberra where over 30 Members of Parliament attended to hear more about the needs of the 6 – 8% of Australian population affected by rare diseases. The function was addressed by Mr. Steve Irons MP (Chair, Standing Committee on Health); Ms Megan Fookes (Executive Director, RVA); Prof. Alistair Kent OBE (Chair, Rare Diseases UK); Ms Kerry Leeds (RVA member) and Ms Kimberlee Francis (MPS Society Australia).

The address from Mr. Steve Irons MP was reassuring from the view that our politicians intend to listen to a united voice representing the needs of those affected by rare diseases.

Prof. Alistair Kent OBE spoke about the recent developments and implementation of a National Plan for rare diseases in the United Kingdom and highlighted the key learning from their rare disease policy that can be reflected in Australia.

RVA has advised that from this event a motion was put forward in Parliament House for the bi-partisan support for a National Rare Diseases Registry and Plan.

PNHSAA continues its support of RVA and its objectives with our secretary Jenny Sturrock currently serving as a board member of RVA.

Mr. Steve Irons MP with Wendy & Michael Patterson

2015 Rare Disease Day, Melbourne

Rare Disease Day, Melbourne

Chhean Khoun, Committee member of the PNHSAA attended the 2015 Rare Disease Day hosted by the Genetic Support Network of Victoria (GSNV) held at the Royal Children Hospital in Melbourne on 27 February 2015. Rare Disease Day is in its 8th year worldwide and celebrated its 6th year in Australia. There were 84 countries worldwide last year and 100 countries this year participating in Rare Disease Day. The main focus for Rare Disease Day this year was collaboration and awareness.

Professor Kathryn North, Director of Murdoch Children Research Institute discussed current research projects and initiatives at the institute. For 2015 there are over 8,000 rare diseases or genetic disorders affecting over 2 million Australians. As a result the Institute's main focus is in collaboration with other disorders in Australia and worldwide and creating initiatives in community through research and support to benefit all patients.

Ms Sue White, Clinical Genomics at Victoria Clinical Genetics Service discussed what is new with Genomic research. Particularly Ms White discussed the research into how using genomics testing methods can quickly diagnose patients with rare genetic diseases. As result patients are diagnosed 54% faster to conventional testing.

For more information on the day or on the GSVN please go to http://www.gsnv.org.au

Share your story

Wendy Patterson

Wendy Patterson was a founding member of the PNHSAA. She is married to Michael who is the current Public Officer/Treasurer of our Association.

In 1994, at the age of 34 and with two young children (Rob aged 6 and Kate aged 4) I was diagnosed with Aplastic Anaemia. This was a very distressing time for our family as we learnt that my bone marrow had been severely damaged and compromised by this illness.

I remember thinking whilst lying in hospital "Please please just let me live to see my children grow up". My husband, Michael was a tower of strength at this difficult time and fortunately I was one of the lucky ones that responded well to treatment for AA.

During the next 12 months I slowly recovered and was eventually back to leading a relatively full and busy life. I began working part-time as a Medical Receptionist and was always involved in family life. I joined School Committees, supported my children's sporting activities and enjoyed being a calisthenics Mum for Kate.

Nine years later in 2003, I was diagnosed with PNH. and discovered that in fact up to 33% of people with a history of Aplastic Anaemia go on to develop PNH.. When I learnt I had PNH. I became enormously concerned about my future. I read what I could on the internet and corresponded with patients in the USA on their PNH website as there was no support available in Australia. Blood transfusions became more regular as I began to suffer many of the common PNH symptoms. Once again however I was to be very lucky. I was referred to Prof.

Jeff Szer who excitedly told me about a trial for a new

drug to treat PNH patients. My timing was perfect and in July 2005, I was enrolled in the Shepherd Trial and began receiving Soliris. My life changed instantly for the better. No more blood transfusions and only a few minor fatigue issues to deal with.

Now, nearly 10 years later my fortnightly visits to the Royal Melbourne Hospital for infusions have just become part of my life. It is a small inconvenience given that

Wendy (middle), husband Michael (left), daughter Kate (left) and son Rob (right)

Soliris has enabled me to live such a full and rewarding life. I have continued to "watch my kids grow up" and be involved in their lives. They are now 27 and 24 and have full time jobs after graduating in Commerce & Economics (Rob) and Chemical Engineering & Commerce (Kate). The enjoyment I have experienced through their lives is so much more than I could have imagined when I lay in that hospital bed 20 years ago!!

Michael and I love to travel and have enjoyed many overseas trips strategically planned between doses of Soliris! Our family enjoys regular trips to the football to watch the mighty Geelong Cats and I still love my work as a Medical Receptionist. Three years ago we moved house and I have been kept very busy 'project managing' several home renovations!

My health has been particularly good since receiving Soliris and so really, I am one of the lucky ones!!

The Committee thanks Wendy for her continued support and sharing her story. To share your story for our next newsletter, email us at info@pnhsaa.org.au.

Update: Review of the Life Savings Drug Program (LSDP)

During November 2014, the Department of Health sought feedback from patient groups, clinicians and Industry on their terms of reference for the review of the Life Saving Drug Program (LSDP) – under which Soliris is funded.

During September 2014 Jenny Sturrock attended an information session facilitated by Rare Voices Australia, that brought together representatives from a number of rare disease groups (most of whom have drugs approved on the LSDP) and representatives from the Department of Health. During this meeting, the Department explained why the review was taking place and reassured patients that it was not the intention of the Department to place new restrictions on accessing drugs currently funded through the LSDP.

Following the meeting, the PNHSAA formulated a response to the LSPD review that focused on our appreciation of access to Soliris highlighting the changes it has brought to PNH patient's lives. We also asked for fairer access to life saving therapies and that little time is wasted between PBAC recommendations and patients gaining access to the drug. We also asked that flexibility around changes to dose timing/volume be in the hands of Specialists and their patients as they keep 'tweaking' things to ensure individuals reach the best outcome possible.

During March 2015 Eve Mulder and Jenny Sturrock will be attending a 2-day summit hosted by Rare Voices Australia that aims to look at what action needs to be taken for a National Rare Disease Plan to be implemented that will address things like equitable access to drugs for those with rare diseases, access to coordinated care, and research into rare diseases is carried out and optimised.

Committee spotlight EveMulder, Committee Member

Eve is our newest PNHSAA committee member. Eve was diagnosed with PNH in her teenage years. In Eve's own words, the PNHSAA has provided her with priceless information and support, as well as a feeling of community and belonging. Eve hopes that by joining the committee, she may give back to the PNH community she has received so much from already. Aside from PNH, Eve is a practicing registered nurse residing on the Mornington Peninsula.

Eve loves the beach (having grown up by the seaside), travel and entertaining and cooking for her friends and family. Eve feels blessed to have such a loving and supportive family unit, her Mother and Partner are also members of the PNHSAA. Eve is enthusiastic about her new role within the PNHSAA and is looking forward to seeing what the future holds for her in the year 2015.

Get Involved : Calling all volunteers

At times information cannot be readily available or updated and events may not be organised as timely as the Association would like. We appreciate the support and assistance of volunteers to aid us in bringing to our members the information, material and events you have asked for. Your support can be as minimal or as 'hands on' as you would like. Please contact us at info@pnhsaa.org.au to become a volunteer.

Team Support : Become a member

Become a member and support the Association which aims to help connect Australians affected by Paroxysmal Nocturnal Haemoglobinuria, increase awareness and understanding of the disease, and advocate best possible care for patients in order to reduce the confusion and isolation that comes with a PNH diagnosis. To become a member, email us at info@pnhsaa.org.au.

Hello : We'd like to hear from you

The PNHSAA would like to hear from its members with suggestions or fundraising ideas, newsletter stories or other charity events the Association can get involved in. Email your suggestions at info@pnhsaa.org.au.

Power of money: Donations are welcome

As we are a small Association we have very limited funding to assist us in providing support services, information and hosting educational and social events for our members around Australia. We appreciate your support and donations to PNH Support Association of Australia Inc. BSB 013-423 Account No:2 545-47305

The big T: Thank you

The Committee wishes to thank all of its members which are made up PNH patients and supporters for all the support, encouragement and donations made to the Association this financial year. We look forward to evolving and growing our community.

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Contact Details

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