Supporting Australians with Paroxysmal Nocturnal Haemoglobinuria

RARE BUT REAL

NEWSLETTER ISSUE №6 MAY 2016

pnhsaa | PNH Support Association Australia Inc

Information

Rare Disease Summit Communique

During the March 2015 Rare Voices Australia summit one of the main aims for bringing rare disease groups and agencies together was to form an in-principle agreement to a summit communique.

The communiqué lists the key principles and objectives of a National Rare Disease Plan and features all of the logos of organisations that have formally endorsed the National Rare Disease Plan. When advocating at Government level for the needs of rare disease patients and the need for a National Plan, Rare Voices Australia uses this communique to demonstrate the level of support in the community.

What is a National Plan? Briefly Australia needs a coordinated, national approach to rare diseases and the correct method of achieving this is through a National Rare Disease Plan. Currently without a plan research and equitable access to treatment (among other things) in the rare disease space are reported as being fragmented which could be indicative of inefficient and ineffective practices. In addition to this the capacity to develop evidence-base, supportive care practices and enhance service delivery to patients is not being done due to a lack of national coordination. As part of the rare disease community PNHSAA wishes to see all patients experience best practice care and access to qualified Specialists to ensure they are achieving the best levels of health they can. This is why PNHSAA is proud to be one of the organisations who have signed the communiqué that is assisting Rare Voices in lobbying for a National Plan.

For more information please use the following link:
https://www.rarevoices.org.au/page/57/communique

Event Spotlight

Annual General Meeting 2015

The Annual General Meeting (AGM) for 2015 was held in August in Melbourne and provided the opportunity to reflect on another year at PNHSAA. Particularly the Committee's work in relation to the Life Savings Drugs Programme Post Market Review Issues Paper. Further, reflecting on those efforts made to form and strengthen ties with other rare disease groups including affiliation with Rare Voices Australia.

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 2016 are:

President: Mathew Dean

Treasurer: Michael Patterson

Secretary: Jenny Sturrock

Committee Representatives: Grace Dean, Chhean Khoun & Eve Mulder

Farewell to Friends

Grace and Mathew Dean

Grace and Mathew Dean have announced that both will be stepping down from the Committee in May 2016 and leaving Australia to embark on a new adventure in Europe and the UK.

Grace has been part of the PNHSAA since its incorporation in 2009 and also joined the Committee at this time. Her enthusiasm and passion to join the Committee stemmed from wanting to provide a hub where PNH sufferers and supporters can obtain relevant information, meet and share experiences with each other. She was a contributor in lobbying for Soliris which she says has been one of the most challenging experiences but invaluable one, speaking out and meeting other PNH sufferers and supporters.

Both Grace and Mathew embarked on the adventure in July 2010 to Washington DC alongside other PNHSAA Committee members to attend the AA&MDS conference to represent the PNHSAA, creating ties with international support groups and obtaining information not only for PNH but also how support groups operate and work internationally.

Mathew joined the Committee and was elected President of the PNHSAA in August 2011. While the position was by no means a small feat, he was enthusiastic about the challenges of the role and encouraged by what the PNHSAA could provide to its members.

Grace and Mathew have immensely enjoyed their time on the Committee – Mathew easily fitting into the role as President with his 'project management' skills and Grace cruising as the 'process driven editor of correspondence and documents'. Most of all they have wish to emphasise the wonderful friendships they have made with all those associated with the PNHSAA and in particularly with Jenny, Chhean, Michael and Eve who they describe as passionate, dedicated and wonderful people who make up the Committee.

Grace and Mathew wish to encourage members of the PNHSAA to join the Committee and continue the great work with Jenny, Chhean, Michael and Eveand become a part of developing a strong platform of support diagnosed with PNH and their supporters.

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A few words

A message from the President

A very big hello to all our members, we are now well and truly into the New Year with Easter been and gone and the AFL and NRL seasons in full swing and with that the PNHSAA committee has already been hard at work planning out 2016.

This first edition newsletter of 2016 newsletter is packed full of interesting articles and to kick off we are pleased to announce that we will be sending out expressions of interest for this year's proposed Members Meeting earmarked to be held in October this year, so please look out for the relating correspondence.

There will be a few changes this year with the PNHSAA committee as I will be stepping down as President and my wife Grace Dean as committee member. We have decided to move to the United Kingdom (and yes we know its cold, grey and get's dark at 4pm in the Winter!), it was a hard decision for us both and we are both sad to leave the committee however excited for its future as Michael, Jenny, Chhean and Eve will continue to do great work and I wish them all the very best.

As such this will be my last President's message however you will be in the capable hands of Chhean Khoun who will be acting President until the general annual meeting in October 2016 where the committee will be calling for nominations and I do encourage you to all consider joining regardless of your location in Australia as video conferencing now allows us to connect to whoever wherever they are.

A reminder that is you haven't renewed your membership subscription yet to please do so as we need as much support as possible to ensure the continuation of this great committee.

Finally thank you all for your ongoing support of the committee and both Grace and myself, we have meet wonderful people during our time on the committee and we are grateful to be a part of the PNH community.

Matt Dean, President

Information

RVA's Rare Disease survey results

Many of our members will recall back in 2014, an opportunity to participate in the online Australian Rare Disease Survey (for adults). Jenny Sturrock and Eve Mulder attended the Rare Disease Summit in Melbourne last year in which the survey results were discussed.

The findings suggest that policy and planning opportunities exist in areas such as patient access to timely and accurate diagnosis, the structure of health services to meet the needs of people living with rare diseases and the collection of evidence to understand the impact of rare disease on the health system.

Over 810 responses were received with 191 rare diseases identified. Half of the respondents said they had received an incorrect diagnosis and 30% said it had taken 5 or more years to obtain a confirmed diagnosis. Participants identified that getting a timely appointment was a problem and contributing factor in delayed diagnosis.

Some opportunities in this space that were identified include:

Treating people as a whole person (not just seeing different Specialists for different aspects of the condition that do not talk to each other); the health for patients who live in remote locations; E-health to facilitate patient records being shared between Specialists, GPs and allied health more easily.

For more information: Rare Voices Australia filmed the Summit and will soon be making available each of the Summit's presentation on their website - https://www.rarevoices.org.au/

A Growing Family

Jenny & Lucas Sturrock

Jenny and Lucas Sturrock are pleased to announce the arrival of their son Flynn John Sturrock born 11 November 2015, weighing 2.5kg. He is now 5 months old and is happy and healthy little man who sister Annabell is so proud to call her brother. All of us on the Committee are so happy for both Jenny and Lucas and we are enjoying cuddles with Flynn at meetings. We wish the Sturrock family love and happiness. Congratulations!

Cody, Ebony & Raiden McKnight

Cody McKnight and his finance Ebony are over joyed to announce the safe arrival of Raiden Jaye Samuel McKnight. Raiden was born at 33weeks 10 September 2015 at 4:13pm, weighing in at 5lbs 3 ounces and 45.5cm long. Cody is a valued member and friend of the PNHSAA. We thank Cody for sharing his amazing news and the Committee wishes the family a happy and healthy journey ahead. Congratulations!

Share your story

Jon Pendse

The perils of being a 19 year old uni student on a night out on the Brisbane river, lets say I must have had a good night up until the boat docked. My night came to an early end when I fell off a bar stool knocking my head and was taken to hospital. I woke the next morning to find out I had an unusual blood count and they wanted to keep me in to check my levels. Two days later my blood counts had not improved but my hangover had passed so I was allowed to go home after being referred to a Haematologist. Up to this point and even to this day, my health has been very good and I have really only had one PNH "episode".

Looking back on my clinical notes I was formally diagnosed aged 19 with aplastic anaemia complicating paroxysmal nocturnal haemoglobinuria. However it was the aplastic anaemia (hypoplastic myelodysplasia) that I was treated for, as my platelet count was severely depleted with counts in the single digits. I was treated with intraveneous Anti-thymocyte globulin (ATG) over 5 days and for the next 12 months, on immunosuppressive medications Cyclosporin and Bactrim. I have been off all medications since 2002. My blood counts have been mostly stable since that time so I have not required a bone marrow transplant, which would have been the alternative should the treatment not have worked.

During the 12 months of treatment, I was advised to reduce my level of gym work as I had been fairly active lifting weights for fitness and muscle building. Within a year or two I had stopped the gym and returned to running which I had done during high school when I competed as an under 16 national state cross country runner.

Life continued in good health with stable 6 monthly blood counts. My wife and I relocated to Perth in 2007 with work and I was referred to a new specialist in WA (Dr Webb), previously under Dr. Morton in Qld. I started seeing my new specialist but only once a year, which then became less frequent due to complacency because I was feeling fine.

In 2013 I experienced my first classic PNH episode including the coke coloured urine. This was a shock having never "experienced" PNH previously. In fact I had forgotten I actually had PNH as I had thought I had aplastic anaemia. As I did not connect my symptoms with Aplastic Anaemia I ended up admitted to Emergency on my GP's recommendation as we did not know what it could be. After tests and discussions with doctors I ended up in haematology, who quickly realised in fact it was my diagnosed PNH.

The 2013 "episode" has been a one off (for now) and I remain in good health. I am now back to three monthly reviews with my new specialist Dr Cull, and am on the "watch & wait" approach. PNH is so diverse affecting everyone in different ways. In many ways I am lucky that PNH has not taken over my life. I am also lucky that should or when PNH becomes "more" active I would be a likely candidate to be approved for the medication Soliris.

Since diagnosis at age 19 (now aged 33); I have achieved many milestones including getting married, having two healthy children (Josef 6 and Ella 5), graduating with a Bachelor of Commerce and also continuing studies to become a qualified CPA accountant. I have been working as an accountant for a major construction firm for over the last 12 years. I have also continued running, completing 39 marathons, my personal best time of 2.48.38 with 16 marathons under 3 hours, 3 ultra marathons and 20 half marathons (pb 1.18.37)! My 40th marathon is scheduled for Perth City to Surf at the end of August 2015. After my 2013 episode I have felt ongoing mild fatigue, my running has slowed a little, but the last 6 months I have continued to improve my running endurance, I may even still have some sub 3 hour marathons left in me yet !

Front and centre in the 2012 city to surf (I finished 11th overall).

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

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

In late 2014 and early 2015, 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.

Following this 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 requested a farer access to life saving therapies and that little time is wasted between PBAC recommendations and patients gaining access to the drug. We also requested 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.

The public consultation on the LSDP Review Issues Paper closed in May 2015 and we will continue to update our members with any updates and new information.

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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