PWS PRADER-WILLI SYNDROME ASSOCIATION NEW ZEALAND
ADVOCACY · EDUCATION · SUPPORT

AUGUST 2025 NEWSLETTER

PWSA(NZ) Committee News

We were pleased to welcome Sarah Verran to the Committee at our Annual General Meeting in June. Sarah has a background in marketing and extensive experience of disability sector governance and management. She lives in the Waikato and is mum to a young adult daughter who has acquired PWS symptoms following a brain tumour. At a more recent Committee meeting we were also pleased that Shelley Mitchell could join us. Shelley lives in Christchurch, is mum to 11 year old Dekken, and she has been considering joining our Committee. Shelley is a strong advocate for Dekken and PWS awareness, and she is very keen to generate more understanding of the syndrome.

The Committee recently held a strategic planning meeting where we set our goals for the next 5 years. We will be sharing our Strategic Plan 2025-2030 with you all soon. We have set ourselves a number of ambitious projects which various subcommittees will be working on, and they may seek your help - watch this space! If you see a particular project that you are keen to sink your teeth into, please sign up!

PWS Clinical Trial Opportunities for Participation

TEMPO™

Pitolisant - TEMPO Study

We'd like to remind you of the opportunity to participate in this phase 3 trial of pitolisant with funded travel to Australia. Marketed as WAKIX and approved in the US and Europe for patients diagnosed with narcolepsy, pitolisant is a potential treatment for EDS (Excessive Daytime Sleepiness) in PWS, which may also provide other behavioural benefits.

Pitolisant is a selective histamine 3 receptor antagonist and it is believed that its activity at H_3 receptors increases the synthesis and release of histamine, a wake promoting neurotransmitter. It has been hypothesised that some of the neurotransmitters dysregulated in PWS are regulated by the histamine 3 receptor (H3R) located in the brain and gut, regulating sleep/wake states, hunger, cognition, and movement.

TEMPO is a randomised, double-blind, placebo-controlled trial with an open-label extension period. The treatment period will be 11 weeks (pitolisant or placebo), with eligible participants having the option to continue in the extension period. Study participation may take around 4-6 months overall with about 5 site visits.

To participate, PWS patients **must be 6 years of age or older** and experience EDS. The trial is mainly evaluating pitolisant's ability to treat EDS, but will also evaluate impacts on severity of irritability, severity of disruptive behaviours, and hyperphagia. For further info about theTEMPO trial: www.youtube.com/watch?v=do0FkvDg6eA

Adults
- Royal Prince Alfred Hospital, Sydney.
Clinical Trials Manager, Tegan Picone, tegan.picone@sydney.edu.au

Children (under 18)
- Sydney Children's Hospital, Randwick site and Westmead site
Lead Study Coordinator, Silvia Hudakova: silvia.hudokova@health.nsw.gov.au and cc the Sydney Children's Hospital study centre: schn-schclinicaltrials@health.nsw.gov.au
- Queensland Children's Hospital, Brisbane.
Study Coordinator, Megan Angliss, chq_pwsresearch@health.qld.gov.au

ARD-101 - HERO Study

We are very pleased to let you know that Aardvark will be opening registrations for their phase 3 HERO trial sites in Australia very soon and discussions have been positive about travel funding for participants from New Zealand. We will be able to confirm this soon. There will be a PWRFA webinar launch when recruitment opens, which we will share the details of.

ARD-101 aims to stop hunger by targeting receptors in the gut to release CCK, a satiety hormone. In PWS there is a deficiency in CCK release because levels do not rise after eating as they would normally. In addition to causing hunger, the deficiency of CCK is thought to exacerbate anxiety, inflammation, constipation and % body fat. During 28 day phase 2 trials, ARD-101 was successful in significantly reducing hyperphagia scores and clinical signs of reduced anxiety and inflammation were also observed. DEXA scans also revealed notable early improvements in body composition.

Australian trial site inclusion and exclusion criteria to be confirmed, but HERO participants will **need to be 13+**, able to commit to the trial for 12 weeks, and attend approximately 4 in-person clinic visits. Participants will have the option to participate in an extension study where everyone receives the drug for a year. Further trial info here: www.heroforpws.com

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Guanfacine for agitation and aggression in PWS

We also have good news about guanfacine coming to New Zealand soon! Guanfacine is already approved by medicines authorities in the USA, Europe, UK and Australia for the treatment of ADHD but is used in PWS for behavioural disturbances such as impulsivity, agitation, aggression and skin picking. Dr Deepan Singh has published several studies on the use of guanfacine in PWS and believes it has a much more favourable side effect profile compared to the antipsychotic medications frequently used in PWS for behavioural symptoms. Dr Singh does not recommend use of typical stimulant ADHD medications in PWS, and guanfacine differs because it is a non-stimulant medication.

Takeda Pharmaceuticals submitted an application to Medsafe for approval in NZ almost one year ago. The approval process can take up to a maximum 18 months, but as a medication already approved by multiple international authorities, we hope to hear an approval decision soon.

Takeda have also submitted a funding application to Pharmac which is awaiting review by the Mental Health Sub-committee. We will also re-submit our own funding application to Pharmac's Therapeutic Group Manager, but some voices of lived experience would be very useful to add to this. If you have experience of behaviours that you think could have / could be treated by guanfacine, please consider sending us a brief summary or story detailing the impact on the person living with PWS, their family or support staff.

IHC Friendship Programme Reminder

In July we held an ALPWS Leadership meeting with guest speaker, Hannah Verry, IHC's Friendship Programme Coordinator. Friendship matches can be in-person (Hamilton, Wellington, Christchurch, Dunedin and Invercargill) or online (nationally). Currently there is only the online option in Auckland due to a waitlist for the in-person programme.

Following the meeting, Hannah asked us to share her contact details. Please get in touch if interested: Hannah.Verry@ihc.org.nz | 022 015 7166

For those in Christchurch who asked, your in-person coordinator is Diana Shoppee: diana.shoppee@ihc.org.nz | Phone: 027 531 3579

DIARY DATE: **Next ALPWS Leadership Meeting** - Monday 8th September from 7pm.
All adults living with PWS are welcome. Zoom link and details will be sent out next week.

New Decision-Making Resource

Whaimana is a new digital resource about supporting people to make decisions. It contains resources, ideas and examples to help decision-makers, family, whānau, Guardians, support staff and advocates to practice supported decision-making and comply with Article 12 of the UN Disability Convention.
The Whaimana website contains a wealth of information: www.supportmydecisions.nz

It includes guides on a range of topics such as how to choose the right supporter for decision-making, considering what is important to the decision-maker, the importance of Māoritanga, understanding the right to take risks, safeguarding, and making the decision.

New PWS Resource Alerts

Report of the
IPWSO
International Prader-Willi Syndrome Organisation
Mental Health Network

Improving mental health and well-being for people with Prader-Willi syndrome
an IPWSO special initiative
www.ipwso.org

IPWSO Mental Health Network Report: "Improving mental health and well-being for people with PWS"
This report aimed to record the collective knowledge and considered opinions of the IMHN, including areas where perspectives may vary. Rather than presenting a single prescriptive approach, it provides a rich resource that reflects the complexity and individuality of mental health in the context of PWS. It is the product of extensive collaboration between parents, clinicians, researchers, care providers, and adults with lived experience of PWS.

www.ipwso.org/information-for-medical-professionals/mental-health

Dr Deepan Singh & Dr Maryssa Portelli - PWS Mental Health
Dr Deepan Singh is a psychiatrist internationally recognised for his expertise in PWS and particularly known for his studies of guanfacine as a treatment for agitation and aggression in PWS.
Dr. Maryssa Portelli is a lead psychiatrist at the PWS Clinic and Emergency Psychiatry Service at RPA Hospital in Sydney.

In this video Maryssa talks about how patients in Australia are accessing guanfacine, the importance of evaluating PWS patients for physical illness which can cause delirium when they first present to ED for

psychiatric evaluation, and Deepan and Maryssa discuss psychosis and some of the psychiatric medications often prescribed to patients with PWS.
Watch here: www.youtube.com/watch?v=WvZiVFHJEIA

Presentations from IPWSO's United in Hope Conference in Phoenix, Arizona. This international conference was hosted by IPWSO, PWSA(USA) and FPWR and held in June 2025.

Family Conference videos can be viewed via a PWSA(USA) YouTube playlist >
We recommend watching Dr Laura de Graff 's presentation on Aging and PWS, Dr Deepan Singh on "Management of Behavioural Problems in PWS", and Dr Jennifer Miller's presentation on endocrinology issues.

The large range of interesting Professional Providers Conference presentations can be viewed via an IPWSO webpage >
They include: Minimising Behavioural Outbursts, new resource 'Practical Portions for PWS', poster presentations on Transition to Adulthood, addressing caregiver staffing shortages, the development of a PWS Specific Respite Service, Effective Communication with People with PWS, Grief in Residential Settings, Aging in PWS, Promoting Self-Advocacy, Relationships in PWS.

IPWSO Professional Caregivers' Forum Update

The last professional Caregivers' Forum took place in May on the subject of ***"Embracing Cultural Diversity in PWS Residential Care."*** Click here to read the meeting notes >
In June there was a bonus Forum meeting as part of the IPWSO United in Hope Conference in Phoenix. The topic was ***"Supporting individuals with PWS in care settings that use less restrictive approaches."*** IPWSO have provided a pdf of the presentation and a summary of the discussion.

The next Forum meeting will be Thurs 25th Sept 8pm UK (**FRI 26TH SEPT, 7AM in NZ**) on the topic of ***"How to establish a PWS residential home."*** This will be a very useful session for service providers looking at establishing new PWS specific homes. Internationally, there will be unique local challenges, but this forum will be used to map training needs, share proven approaches and identify varying funding systems across the regions. It is an opportunity to spotlight recent global successes and learn directly from those on the ground. Whether you're just starting out or already running a group home, your experience matters. The plan is for the meeting attendees to explore scalable models and support each other in developing sustainable, person-centred care.

We would like to encourage residential services and support providers to join the PWS Caregivers' Forum, an excellent opportunity to connect services both nationally and internationally. Anyone working in a paid role supporting people with PWS is welcome to join. The Forum is supported by the Professional Providers and Caregivers Board (PPCB) of IPWSO, an advisory board dedicated to sharing best practice, information, knowledge and techniques to those providing care to the global PWS community.
Click here and scroll down for further information and to join the forum >

IPWSO are also keen to hear from members wishing to contribute to the forum, and this can be done in whatever ways are most comfortable. The Forum is for PWS Caregivers, by PWS Caregivers so you are welcome to help shape the advice and information that comes out of the Forum. Some of the ways you may be able to contribute are:

- writing to them about residential care in New Zealand
- contributing an article, blog, useful documents or videos
- requesting to present as part of a Forum meeting

With any questions or contributions, contact Manager Shelly Cordner: scordner@ipwso.org

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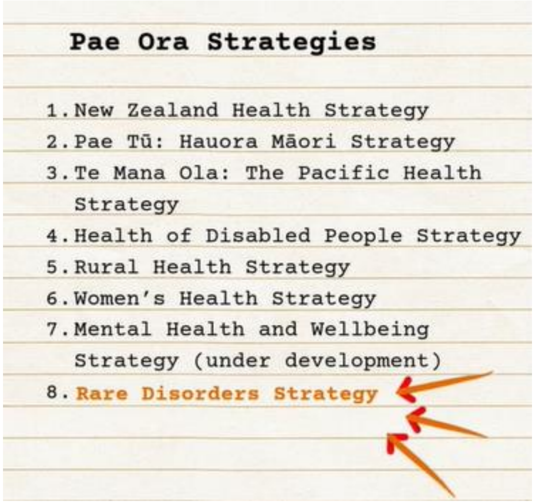
Sibling Advisory Group

Researchers at AUT are working on projects relating to siblings of children/young people who have a long-term health condition. They are developing a sibling advisory group who will shape ongoing research.

This is an opportunity for siblings (16-24 yrs) to be research partners and provide their opinion about what type of research, resources or supports they would find helpful. (Please note, this is not recruitment for a research study.)

It would be fantastic if siblings of people living with PWS were to have input into future research projects. To register or ask questions, please click here for the registration form >

Implementation of a Rare Disorders Strategy



Pae Ora Strategies

1. New Zealand Health Strategy
2. Pae Tū: Hauora Māori Strategy
3. Te Mana Ola: The Pacific Health Strategy
4. Health of Disabled People Strategy
5. Rural Health Strategy
6. Women's Health Strategy
7. Mental Health and Wellbeing Strategy (under development)
8. Rare Disorders Strategy

The Government is planning to make a number of amendments to the Healthy Futures (Pae Ora) Act and RDNZ is once again calling for the Rare Disorders Strategy to be incorporated as one of the official health strategies in the legislation. Click here to read RDNZ's submission >

If you haven't already, please sign and share RDNZ's petition demanding action on the Rare Disorders Strategy: rdnz.nationbuilder.com/petition

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Dates for your Diary

Wellington Transition Expo 'What's Outside the Box?' - Tues 2nd September
Auckland Transition Expo - Weds 3rd September, 9am - 2pm
Next ALPWS Leadership Meeting - Mon 8th September @ 7pm
Next IPWSO Caregivers Forum - Fri 26th Sept @ 7am

Contact Us:
www.pws.org.nz | enquiries@pws.org.nz | 0800 4 PWS Help