

## March 2025 Newsletter

### Family Support Camp 2025



The sun was shining again for our family support camp, and a great time was had by all. There were 94 people registered this year, our biggest camp yet! If you came along and haven't completed feedback yet, please consider doing so using the [link](#) sent via email. We have already started to plan our next camp! Click here to [view photos and read more >](#)

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### Historic Approval of First Hyperphagia Treatment



You have probably heard the exciting news that the FDA in the US have just approved VYKAT XR (previously known as DCCR) for hyperphagia in PWS. This is not only the first treatment approved for hyperphagia, but also the first treatment approved for PWS since GHT in 2000!

VYKAT XR has been approved for adults and children with PWS 4 years and older. It is administered by extended-release tablets taken once daily. It is thought to work by

activating potassium channels, which may decrease the secretion of peptides that regulate appetite. Metabolically, it reduces leptin, insulin, and insulin resistance, in addition to increasing adiponectin levels. Significant reductions in hyperphagia were observed in trials, particularly in patients with more severe hyperphagia, and other positive results were achieved, including reductions in fat mass. VYKAT XR was generally well tolerated, but some side effects were seen that can now be monitored whilst receiving treatment.

However, VYKAT XR did not work for everyone in trials and multiple hyperphagia treatments will be needed to treat the complexity of hyperphagia in PWS. This unpredictability of effectiveness may potentially cause issues with Pharmac funding approval when we reach that stage in the process of seeking availability in New Zealand.

### **When will VYKAT XR become available in New Zealand?**

As is typical with new medicines, it will be some time before VYKAT XR will be available here. We are currently waiting for response from Soleno Therapeutics about their plans to seek approval with Medsafe (our equivalent to the FDA), but we think it is likely that Soleno will apply to larger regulatory authorities next, such as the EMA in Europe. We will maintain contact with Soleno to ensure we are kept in mind and that they are aware of our regulatory processes. Once Soleno (or licenced company) sponsors an application to Medsafe, the assessment process could take up to a maximum of 200 days, we can apply to Pharmac for funding of VYKAT, and we would urge clinicians to do the same.

Unfortunately, VYKAT XR is a very high cost medicine (~2330 NZD - 5437 NZD per day at current market price dependent on weight) which means we may have to fight hard to achieve Pharmac funding, but we have done this before and can do this again! Pharmac's decision criteria are not entirely focused on cost-effectiveness, they do have a Rare Disorders Specialist Advisory Committee that we can apply to. Pharmac may be able to negotiate a reduced price for VYKAT (although this is unlikely with no competitive biosimilar product available), or the market price may reduce in time, or we may be able to find other funded access pathways that become available for VYKAT.

We will keep you informed of any progress and news as it becomes available. In the meantime, you may wish to register for the MedLive and Soleno webinar on VYKAT XR taking place @ 4.15am Thurs 17th April NZ time, but don't worry, it will be recorded - just [register here](#) and you should be sent the recording when available.

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## **Growth Hormone Therapy & Disability Allowance**

Growth hormone therapy for adults living with PWS is currently on Pharmac's Options for Investment list and whilst we wait for this to be funded, we want to make sure that families and adults living with PWS are aware of other avenues for accessing treatment.

[Disability Allowance](#) is for regular or ongoing costs due to the disability and can cover things like medicines, prescriptions, doctor visits, medical equipment, and travel etc. In many cases it can cover the cost of GHT, depending on the dose needed. In order to apply, you will need to provide proof of costs, which usually means paying for initial

treatment so that you can show the receipts. (GHT may cost around \$250 per 0.2 dose for approx. 3 months' supply, depending on pharmacy. Some adults may have higher doses of 0.4 or 0.6.) You will also need a doctor to complete part of the application verifying the disability and related costs. If you have any questions, please get in touch.

It may be timely to check that families are also aware of other WINZ benefits available to them if their son or daughter with PWS 16 years or older is living at home. Both the Supported Living Payment and Accommodation Supplement can be claimed at this time. Please let us know if you have any questions.

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## New Zealand Rare Disorders Month



You may have noticed the Sky Tower glowing orange, or other landmarks lit up during March for Aotearoa New Zealand's Rare Disorders Month.

As we united to celebrate rare, the rare community also continued to apply pressure to Health New Zealand - Te Whatu Ora to implement an action plan for the Rare Disorders Strategy. We believe the strategy, a Centre of Expertise, and health pathways / standards of care are desperately needed to improve the health and wellbeing of people living with PWS.

If you haven't already, please sign and share RDNZ's petition demanding action:

[rdnz.nationbuilder.com/petition](https://rdnz.nationbuilder.com/petition)



## Results of international PWS study, including NZ.

Last year several of our members participated in the FAM-PWS study examining the experiences and well-being of families living with Prader-Willi syndrome. There was a great response from families in Aotearoa New Zealand, and the researchers would like to thank you for your participation. The results are [now published](#) and confirm the previously identified high levels of stress amongst parents and siblings of people living with PWS, but this is the first study to do so in a large multi-country sample.

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## PWSA(NZ) Advocacy Updates

We hope many of you were able to submit a response to the DSS consultation on assessment tools and processes, and the suggested options for flexible funding. We also submitted a response which can be [viewed here >](#)

We have also met with the DSS taskforce regarding our concerns around residential care - how the new pricing scheme being developed might affect people living with PWS, whether the residential care needs of people living with PWS are fully understood, the issue with current waitlists, and the lack of PWS specialism. We also raised the issue with the taskforce that there is a need for more PWS specific residential care options and put a case forward for why this is needed. They agreed to read our [proposal document](#) and have passed it onto the commissioning team for review. We will follow this up in due course.

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## PWSA(NZ) AGM - seeking officer nominations

We are aiming to hold our Annual General Meeting towards the end of April, date TBC. This will be an online meeting - keep an eye on your inbox for further information!

Our AGM is an opportunity for anyone interested in becoming a PWSA committee officer to put yourself forward for nomination - we would love to hear from you! We are looking for keen, enthusiastic people who feel they have skills to offer.

Our Committee meets once every 2 months, usually online. Various roles are available and tasks can be shared amongst committee members or project sub-committees. Please [get in touch](#) if interested.

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## IPWSO Caregivers' Forum Update

The last professional Caregivers' Forum took place in January on the subject of *"The Experience of Grief for Residents and Staff in a PWS Residential Setting."* This meeting was attended by our Caregiver Delegate and PWSA(NZ) committee member, Julie-anne Quinney, and [meeting notes can be viewed here](#).

Julie-anne would like to encourage others to join the forum which is an excellent initiative to connect residential support providers, both nationally and internationally.

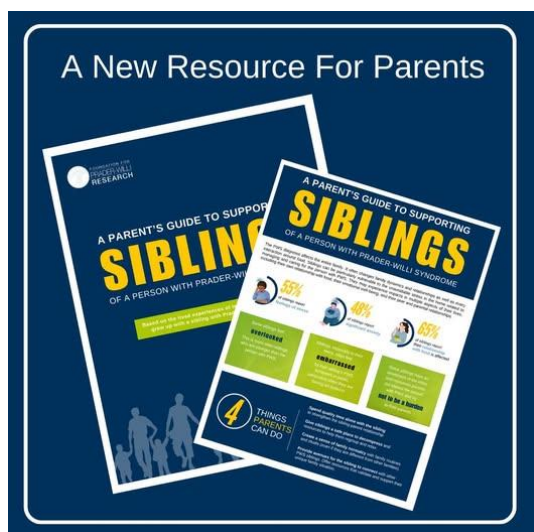
The next forum meeting will be Thurs 22nd May @1pm UK (**Friday 23rd May at 1am NZ time**). There are 3 meetings per year held at different times to suit time differences. The next topic will be *"Embracing Cultural Diversity in PWS Residential Care."* Click here for more [topic information and to join the form >](#)

- Forum newsletters following meetings
- A Forum webpage which includes latest news and shared resources
- Ad-hoc support for networking in your own country

After joining, you will receive invitations to meetings, newsletters and shared resources.



## New PWS Resource Alerts



### A Parents Guide to Supporting Siblings

New resource from the FPWR. Siblings can be negatively impacted by family stress related to managing and caring for someone with PWS, particularly when behavioural challenges are more prominent.

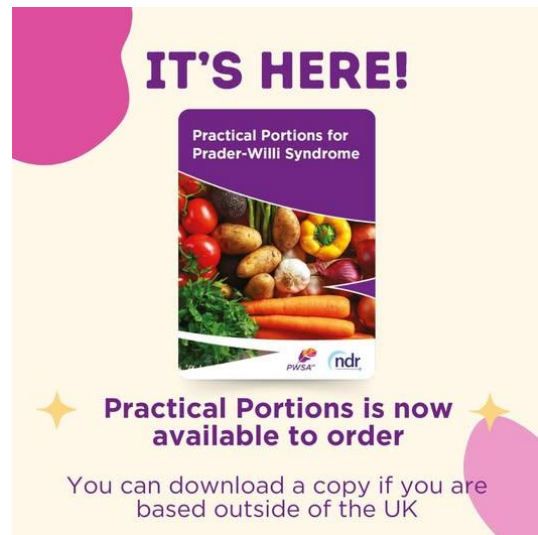
This guide can help parents understand the challenges faced by [siblings](#) and provides helpful advice and resources to support them. Download your copy here: [www.fpwr.org/supporting-siblings](http://www.fpwr.org/supporting-siblings)

### Practical Portions for PWS

This is a useful new guide available from PWSA UK. Practical Portions was created by dietitians and nutritionists specifically for the PWS community, designed to make it easy to work out appropriate portions.

Freely available to download here:

[www.pwsa.co.uk/practicalportions](http://www.pwsa.co.uk/practicalportions)



### Dr Maryssa Portelli - Guiding Mental Health Presentations in the Emergency Department

This is a must-watch session!

Dr. Maryssa Portelli is a staff-specialist psychiatrist at the RPAH PWS Clinic and Emergency Psychiatry Service in Sydney.

Watch here: <https://youtu.be/iwXRwgoEvY>



### Dates for your Diary

**PWSA(NZ) AGM** - April date TBC

**PWS Awareness Month** - May 2025

**Next IPWSO Caregivers Forum** - Friday 23rd May, 1am.

**PWS Awareness Day** - Friday 30th May

**IPWSO Conference, Phoenix, USA** - 24th - 28th June, 2025

**Christchurch Transition Expo 'Next Steps'** - Friday 4th July 11am - 4pm

**Wellington Transition Expo 'What's Outside the Box?'** - Tues 2nd September

**Auckland Transition Expo** - Weds 3rd September, 9am - 2pm