Prader Willi Syndrome
Association Ireland
Carmichael Centre
Carmichael House
North Brunswick Street
Dublin 7
IRELAND

info@pwsai.ie
www.pwsai.ie

Reg. CHY number:15171
CRA Reg. 20051693
Company Registration: 364058

31st March 2025

Important News for the PWS Community

On the 27th March 2025, VYKAT™ XR (diazoxide choline), formerly known as DCCR, was been approved by the U.S. Food and Drug Administration (FDA) for the treatment of Hyperphagia in adults and children (ages 4 and older) with Prader-Willi Syndrome (PWS). This is a significant step forward, as VYKAT XR is the first FDA-approved treatment for Hyperphagia in PWS.

Benefits of VYKAT XR:

- Reduces Hyperphagia – Helps control the intense hunger that is one of the most challenging aspects of PWS
- Supports Metabolic Health – May help regulate insulin and improve fat metabolism.
- Improves Behaviour & Mood Stability – Clinical trials suggest it may help with anxiety, aggression and other behavioural challenges that people with PWS may have.
- First FDA-Approved Treatment for Hyperphagia in PWS – Until now, there has been no specific FDA-approved treatment for Hyperphagia in PWS.

Known Serious Side Effects of VYKAT XR:

- Hyperglyccemia (High blood sugar levels)
- Fluid Overload causing swelling (edema)
- Diabetic ketoacidosis

Availability & Approval Status:
VYKAT XR is currently approved only in the United States and is expected to be available from April 2025 in the USA.

The Road Ahead in Ireland

While this approval is a positive step, it's important to remain realistic about the journey ahead for VYKAT XR in Ireland. The approval process for new treatments is a lengthy one, and the next steps will involve:

- European Medicines Agency (EMA) Approval – Firstly, the medication will need to gain approval from the EMA, which would allow its use across the EU, including Ireland.
- Health Products Regulatory Authority (HPRA) Approval – Once approved by the EMA, the HPRA will assess its safety and effectiveness specifically for Ireland.
- Health Service Executive (HSE) Reimbursement Decision – Following the HPRA approval, the National Centre for Pharmacoeconomics (NCPE) will review the treatment's cost-effectiveness and discussions with the HSE will determine whether it can be publicly funded for patients in Ireland.

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This road will take some time to navigate before we have access in Ireland and it is important to remember that these stages are necessary to ensure the safety of the drug for people with PWS. It is also important to recognise that VYKAT XR may not work for every individual with PWS. There may also be varying degrees of success and it cannot be seen as a cure for Prader-Willi Syndrome.

Also, with only one current supplier the costs are massive. We must stay grounded in our expectations of timelines and also of successful treatment, but also allow ourselves some hope.

The approval of VYKAT XR is a significant milestone for the PWS community, and it may signal the start of more treatments for hyperphagia in PWS in the future.

Much like the long road that led to the approval of Growth Hormone Therapy 25 years ago, this process may take some time. While Growth Hormone Therapy is almost a normality in PWS in Ireland currently, even just 10 years ago it was not as simple to gain access.

We will continue to work with government agencies, Soleno Therapeutics, leading PWS Endocrinologists in Ireland and the wider PWS community across Europe to ensure that treatments like VYKAT XR can become part of the future for people with PWS in Ireland.

Statement on behalf on the Board of Trustees Prader Willi Syndrome Association Ireland (PWSAI)

www.pwsai.ie

Sources of information:

- Miller JL, Gevers E, Bridges N, et al. Diazoxide choline extended-release tablet in people with Prader-Willi syndrome: results from long-term open-label study. Obesity (Silver Spring)
- VYKAT XR website https://www.vykatxr.com/
- Soleno Therapeutics Press release - https://investors.soleno.life/news-releases/news-release-details/soleno-therapeutics-announces-us-fda-approval-vykattm-xr-treat