

CLINICIAN STATEMENT

VYKAT XR™ (diazoxide choline): Safety Considerations for People with Prader-Willi Syndrome

Important information for families considering VYKAT XR for their loved one with PWS

What is VYKAT XR?

VYKAT XR (also called DCCR, or diazoxide choline-controlled release during clinical trial) is a medicine approved by the FDA. It is used to treat extreme hunger (called hyperphagia) in adults and children aged 4 years and older who have Prader-Willi syndrome (PWS). Since it became available in the spring of 2025, more than 800 people have been prescribed this medicine.

How does it work?

VYKAT XR is thought to work by affecting certain channels in brain cells that help control the release of natural chemicals (called peptides). Some of these peptides are involved in hunger. Scientists believe this is how the drug helps reduce extreme hunger, but the exact details are still being studied. The drug can also interact with other medications, so it is important that your doctor knows about all medicines being taken.

What safety concerns have been reported?

The channels in the brain that VYKAT XR works on to reduce hunger are also present in other places in the body. Activation of these channels by VYKAT XR can cause problems due to fluid retention, especially in people who have risk factors for heart failure and breathing problems. Since VYKAT XR became widely available, some serious health problems have been reported to the FDA. These include:

- **7 deaths** (mostly related to heart or breathing problems)
- **A number of cases of other serious heart or breathing problems** that did not result in death

All of the people who died or had serious problems also had other complex health issues, were taking multiple medications, and many were living with severe obesity.

Does this mean the drug caused these problems?

Not necessarily. These types of heart and breathing problems are also common in people with PWS, especially when weight and sleep apnea (a condition where breathing stops and starts during sleep) are not well managed. People with PWS already have a higher risk of death at all ages compared to the general population (estimated at 1-3% per year). Based on these reports alone, it is not possible to say for certain whether these deaths were related to use of the drug or were part of the health challenges that come with PWS.

Why didn't this come up in the clinical trials?

Clinical trials follow strict rules to make sure new treatments are both safe and work well. When a disease is rare, fewer people take part in the trial than would for a more common disease. This means that doctors need to gather further information over time about how well it will work in the real world and its safety in the broader PWS patient population.

In addition, people who are very sick or have other serious health problems are usually not included in clinical trials. This is necessary to keep them safe. As a result, when a new drug is approved, doctors may not know exactly what to expect in patients who have more medical problems and other complications. That is why doctors need to be careful when using new medicines in patients with more complicated medical problems.

Who may be at higher risk for problems?

Certain factors may increase the chance of serious side effects. These include:

- Obesity, especially severe obesity
- Heart or breathing problems (now or in the past)
- Swelling or fluid buildup in the body
- Sleep apnea that is not being treated or not well controlled
- Other breathing issues, such as shallow breathing (hypoventilation)
- History of severe respiratory infections or pneumonia

What should you do if you or a family member is taking or considering VYKAT XR?

- **Make sure your doctor knows PWS well.** People with PWS have unique health needs. For example, they often do not complain about pain or discomfort, which can make it harder to spot problems early.
- **Your doctor should read the drug's full prescribing information** (called the package insert) carefully. It includes important guidelines for safe use.
- **Close monitoring is important.** Your doctor should do safety tests before starting the medicine and continue to watch for any warning signs while you are taking it.
- **Ask for help if needed.** If your doctor does not have a lot of experience with PWS, they should consider reaching out to a doctor who does. The three major PWS patient support groups can help connect you with experienced providers.
- **Read the latest guidelines.** A group of doctors experienced in caring for people with PWS recently published helpful recommendations for using this medicine safely (PMCID: [PMC13148164](#)). The publication is free for you to download and share with your healthcare providers.

Key Takeaway

VYKAT XR can be a helpful treatment for extreme hunger in PWS, but it is important to be aware of the risks, especially for people who have other health problems. Work closely with a doctor who understands PWS and follow all recommended safety steps.

The PWS community is grateful for the continued partnership with pharmaceutical companies developing and testing new drugs for the complex medical and behavioral features of PWS. We welcome new clinical studies focused on safety and efficacy to improve quality of life for families and individuals living with PWS.

PWS Patient Organizations:

Foundation for Prader-Willi Research

<https://www.fpwr.org/>

International Prader-Willi Syndrome Organization

<https://ipwso.org/>

Prader-Willi Syndrome USA

<https://www.pwsausa.org/>