

VYKAT™ XR FAQ FOR PARENTS PRADER-WILLI SYNDROME



Created by

In collaboration with the
PWSA | USA Clinical &
Scientific Advisory Board

Indication / Hyperphagia



**Q: Can this medication be used even though my child does not have hyperphagia?
Can VYKAT™ XR (diazoxide choline) be used to possibly prevent hyperphagia?**

No, VYKAT XR is currently only approved to treat active hyperphagia in Prader-Willi syndrome (PWS)—meaning when a child already shows extreme hunger and food-seeking behaviors. It isn't meant to prevent hyperphagia before it starts. That's a very good question, and it's something researchers might study in the future. Right now, we don't have any evidence that the medicine works for children who don't yet have hyperphagia. The medication is usually considered when you and your doctor notice that your child's interest in food is clearly different from other kids their age. Because VYKAT XR can have side effects, it's important to use it only when the benefits are clear, not before.

Q: Has the drug been tested for effectiveness in different genetic types of Prader-Willi Syndrome?

Yes, the drug was tested in people with different genetic types of Prader-Willi syndrome (mostly Caucasian), like deletion and UPD. It worked well across all these groups.

Q: How long does it usually take before we see a decrease in food-seeking (hyperphagia) with VYKAT XR?

Some families start to notice small changes in food-related behaviors about 6–8 weeks after their loved one reaches the right dose. However, the full benefits often may take longer to appear—sometimes several weeks. Research has shown that it may take about 6 to 9 months of treatment to see the maximum improvement. Every person with PWS is different, so progress can vary.

Q: Can VYKAT XR be used for weight loss?

VYKAT XR is **not approved by the FDA for weight loss**. However, by helping reduce hunger and the strong drive to eat, it may make it easier to manage weight when used alongside a healthy diet, safe food practices, and regular physical activity.

Effectiveness / Monitoring

Q: How does VYKAT XR help reduce hunger or hyperphagia?

VYKAT XR helps regulate certain signals in the brain and other organs that control appetite. In Prader-Willi syndrome, these signals don't work as they should, which leads to constant feelings of hunger. By balancing these signals, VYKAT XR can help reduce the strong food-seeking drive (hyperphagia).

Q: How will we know if it is working?

You and your care team will monitor your child's appetite and food-related behaviors, such as food-seeking or constant focus on food. Some families notice small improvements in the first few months, but it may take up to 6 months (or sometimes longer) to see the full effect.

Parent Tip: It's important to give the medication consistently. At the same time, environmental controls, behavioral strategies, and good nutrition remain essential parts of managing hyperphagia.

Q: How effective is VYKAT XR for managing food-related behaviors?

Clinical studies and early experience show that VYKAT XR can help reduce food-seeking behaviors and preoccupation with food in many children and adults with PWS. Some families notice improvements within the first few months, but the full effect may take 6–9 months of consistent use. Effectiveness can vary from person to person, and the best results are usually seen when the medication is combined with structured meal plans, food security measures, and behavioral support.

Parent Tip: Keep a simple log of your child's food-related behaviors, appetite, and mood. This helps you and your care team see patterns over time and measure progress, so adjustments to treatment or strategies can be made if needed.

Q: Will VYKAT XR help with other behaviors, like tantrums or anxiety?

VYKAT XR is approved specifically to help reduce hyperphagia (the strong drive to eat). However, some families notice that when hunger decreases, their child may also have improvements in mood, frustration, or certain disruptive behaviors. It's important to remember that this medication is not a treatment for anxiety or other behavioral challenges, which should continue to be addressed through behavioral support, therapy, and other recommended interventions.

Q: Who will monitor changes in hyperphagia, and will a questionnaire be used?

Your care team will monitor changes in hyperphagia over time. While a questionnaire is often used in clinical trials, it is typically not used in clinical care.

Side Effects / Safety

Q: What are the common side effects of VYKAT XR?

Common side effects include fluid retention (swelling in legs or face), increased blood sugar (usually early in treatment), and increased body hair. For most people, these side effects are mild and manageable. Some people may be more sensitive and notice stronger reactions. Adverse reactions can sometimes be more noticeable if your child/adult with PWS is taking other medications or supplements, so it's very important to review everything with your healthcare provider or pharmacist before starting VYKAT XR.

Q: Can a person with a history of edema safely take VYKAT XR?

Yes, a person with a history of edema can take VYKAT XR, but it should be done with caution. VYKAT XR can cause or worsen swelling, so your doctor will monitor closely and may adjust the dose if needed. Experts recommend that any existing edema be well evaluated/managed before starting the medication. For patients with significant swelling, a cardiac evaluation may be suggested by the care team before beginning VYKAT XR to ensure safety.

Parent Tip: Request clear guidance. Ask your healthcare provider to provide a specific plan for how and when to check for swelling or fluid overload, including what signs to look for and how to record changes.

Q: Should a pulmonologist/cardiologist be consulted prior to starting VYKAT XR?

You should discuss with your prescribing provider whether a cardiology or pulmonary evaluation is needed before starting VYKAT XR. This is especially important if your child has other risk factors for fluid retention or pulmonary issues, such as severe obesity, severe obstructive sleep apnea, pre-existing peripheral edema, or pre-existing heart conditions. For many individuals without these additional risk factors, these consultations may not be necessary. If your child/adult has existing respiratory or cardiac conditions, or baseline edema, your provider may recommend further evaluation to ensure it is safe to begin the medication.

Parent Tip: Share your full medical history, including any heart or lung concerns, with your care team before starting VYKAT XR. This helps the team determine if additional evaluations are needed for safety.

Side Effects / Safety (cont.)

Q: How often should blood sugar be checked while taking VYKAT XR?

Before starting VYKAT XR, it's important to have a baseline evaluation of your child's blood sugar, including an HbA1c test. Once treatment begins, fasting blood sugar should generally be checked **weekly for the first 2 weeks** and then **every 4 weeks**. HbA1c testing is usually done **every 3 months**. Your doctor may adjust this schedule based on your child's medical history, response to VYKAT XR, or any pre-existing blood sugar concerns. Always follow your clinician's guidance for monitoring.

Parent Tip: Keep a simple log of blood sugar readings to share with your care team during visits. This helps track changes and supports safe, effective treatment.

Interactions / Pharmacology

Q: Does VYKAT XR negatively interact with other medications?

VYKAT XR may interact with some other medications. Most often, interactions happen because two medicines are processed by the liver in similar ways, which can change how strong either one is.

Parent Tip: To keep things safe, it's very important to tell your doctor about **all** medications you are taking—including prescriptions, over-the-counter medicines, vitamins, and herbal or natural remedies. If an adjustment is needed, your care team will guide you.

Q: Is pharmacogenomic testing helpful for dosing or interactions?

Not usually. While VYKAT XR is broken down in the liver by enzymes (CYP1A2 and CYP3A4), routine genetic testing is not needed for most people, and it is not clear that the results would improve safety or effectiveness. Doctors usually adjust the dose based on how each person responds to the medication, rather than on genetic testing. If you have questions about whether pharmacogenomic testing might be helpful for your child, it's best to discuss this directly with your doctor.

Q: Any important interactions with medications commonly used in PWS, like pitolisant, guanfacine, or risperidone?

So far, there is no clear evidence of dangerous interactions with those medications. Your doctor will still review all medications, supplements, and herbal products to ensure safety.

Parent Tip: Bring an up-to-date list of all prescriptions, over-the-counter medicines, vitamins, and supplements to every clinic visit.

Q: When taking SSRIs or atypical antipsychotics with VYKAT XR, is lab monitoring needed?

For most such medications, routine blood level monitoring is not available or considered useful. In the case of SSRIs or atypical antipsychotics taken with VYKAT XR, there is no evidence that regular lab tests would improve safety or effectiveness. Your care team will focus on monitoring how your child is responding to treatment and watching for any side effects.

Q: Is caffeine use discouraged?

VYKAT XR can make the effects of caffeine stronger, which may lead to symptoms like restlessness, trouble sleeping, or a fast heartbeat. Because of this, your daily caffeine intake may need to be reduced. It's best to talk with your doctor about how much caffeine is safe while taking VYKAT XR.

Parent Tip: Remember that caffeine isn't just in coffee—tea, chocolate, sodas, and energy drinks can all add up. Keep track of your child's caffeine intake and share it with your care team.



Q: Is it safe to drink grapefruit juice or herbal teas?

Grapefruit juice can affect how certain medications are processed in the liver, and it may increase the levels of VYKAT XR in the body. Because of this, it is generally **recommended to avoid grapefruit juice** while taking VYKAT XR. Most herbal teas are safe, but some herbs can also interact with liver enzymes or other medications, so it's best to **check with your doctor or pharmacist** before using any new herbal teas or supplements.

Parent Tip: Keep a list of all beverages, teas, and supplements your child consumes and review it with your care team to make sure nothing interferes with VYKAT XR.

Diet / Lifestyle

Q: Is there a special diet required while taking VYKAT XR?

No special diet is required to take VYKAT XR, but following a lower-calorie, high-fiber diet can be helpful. Moderating the amount of raw vegetables can support blood sugar balance, and avoiding sugary snacks and drinks is especially beneficial. VYKAT XR does not cause weight loss on its own; its goal is to reduce the sense of hunger toward a more normal level so that managing diet may feel less stressful for the person with PWS. Eating a healthy diet with appropriate calories to reach and maintain individual weight goals remains critical. It's important to work closely with your doctors and nutritionists to determine the best dietary plan for your child/adult with PWS.

Q: Because many individuals with PWS eat a diet higher in vegetables, should cruciferous vegetables (like broccoli, cauliflower, or brussels sprouts) be limited while taking VYKAT XR?

No, there is no need to avoid cruciferous vegetables. These foods are healthy and safe to eat while taking VYKAT XR. Some studies suggest that certain vegetables may affect liver enzymes (like CYP1A2), but the clinical importance of this is not clear, and there is no evidence that it changes how VYKAT XR works. Cooked cruciferous vegetables are often a key part of a balanced diet for people with PWS, and there are currently no recommendations to restrict them while on this medication.

Administration / Dosing

Q: Can VYKAT XR tablets be crushed or chewed?

No, **VYKAT XR tablets should not be crushed, chewed, or split**. They are designed as extended-release tablets, which means they release the medication slowly over the day. Crushing or chewing the tablet can release too much of the drug at once, which may increase side effects or reduce its effectiveness. Always take the tablet whole, exactly as prescribed by your doctor.

Parent Tip: If your child has difficulty swallowing pills, talk with your care team—they can provide guidance or alternatives without affecting the safety or effectiveness of the medication.

Q: How should VYKAT XR be taken each day?

VYKAT XR is an extended-release tablet and should be taken **once daily**, at the same time each day. The tablet should be swallowed whole with water and **not crushed, chewed, or split**, because this can affect how the medication works and may increase side effects.

Parent Tip: Pick a routine time, such as after breakfast, and try to be consistent every day.

Administration / Dosing (cont.)

Q: What should we do if a dose is missed?

If a dose of VYKAT XR is missed and it has been **less than 7 days**, you can resume your child's usual dose. If it has been **7 days or more**, contact your doctor before giving the medication. Your child may need to restart at a lower dose, and your doctor can guide you safely.

Parent Tip: Keep a simple log of doses and missed days to help you track medication use and make it easier to discuss with your care team.

Q: Can the dose be adjusted at home?

No, the dose should **only be adjusted by your doctor**. Doctors base dose changes on your child's response, side effects, and clinical monitoring.

Parent Tip: Keep notes on appetite, behavior, and any side effects to help your doctor make informed decisions about dosing.

Q: Can VYKAT XR be taken with or without food?

VYKAT XR can be taken **with or without food**, whichever works best. Taking it with food may help reduce mild stomach upset.

Parent Tip: Choose a consistent approach—either always with meals or always without—to maintain steady medication levels.

Storage / Pharmacy

Q: How should VYKAT XR be stored?

Store tablets in the bottle provided by the pharmacy at room temperature in a dry place, away from heat, moisture, and light. Keep the medication out of reach of children.

Parent Tip: Avoid storing in the bathroom or near the kitchen sink, where humidity can affect the tablets.

Q: What should we do if a tablet is dropped or damaged?

If a tablet is dropped or broken, **do not use it**. Give the next full dose at the scheduled time. Contact your pharmacy for replacement tablets if needed.

Q: Can VYKAT XR be taken at the same time as other medications?

Yes, but always share a complete list of all medications, supplements, and herbal products with your doctor, as some combinations may affect how VYKAT XR works.

Parent Tip: Bring an updated medication list to every appointment to help your care team manage safety and interactions.

Q: Can VYKAT XR be filled at a local retail pharmacy?

No. VYKAT XR is not available at all local retail pharmacies because it is a specialty medication. Families receive it through an exclusive Specialty Pharmacy (PANTHERx Rare) which is responsible for the distribution of VYKAT XR. PANTHERx is headquartered in Pittsburgh, Pennsylvania and is licensed in all 50 states and U.S. territories.

Parent Tip: Contact your clinic or PANTHERx ahead of time to ensure the medication will be available when you need it and to avoid any delays in starting treatment.