



Neurocrine Biosciences Announces Publication in Orphanet Journal of Rare Diseases Advancing Understanding of Hyperphagia in Prader-Willi Syndrome

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- Expert perspectives highlight the interaction of hypothalamic dysfunction, biological, behavioral, and environmental factors in shaping the challenges of hyperphagia in Prader-Willi syndrome for patients and families beyond obesity-related complications
- The manuscript underscores the importance of multidimensional assessment and integrated care management across the patient lifespan
- Experts conclude VYKAT® XR (diazoxide choline) extended-release tablets represent a significant advancement in the treatment of hyperphagia, potentially complementing behavioral and environmental approaches

SAN DIEGO, Oct. 22, 2026 /PRNewswire/ -- [Neurocrine Biosciences, Inc.](#) (Nasdaq: NBIX) today announced a publication of expert perspectives highlighting hyperphagia in Prader-Willi syndrome (PWS) as a complex, neurobiologically driven condition that extends beyond excessive hunger and requires comprehensive assessment and management across the patient lifespan. The publication provides insights from an international group of clinical and scientific experts on the evolving understanding of hyperphagia and its implications for the care of people with PWS. The manuscript appears in [Orphanet Journal of Rare Diseases](#).



"Neurocrine is committed to advancing understanding of Prader-Willi syndrome and addressing the needs of individuals and families affected by this complex disorder," said Eiry W. Roberts, M.D., Chief Medical Officer, Neurocrine Biosciences. "Recognizing hyperphagia as a multifaceted, neurobehavioral condition is essential to understanding its real-world impact on people with PWS and their families. By connecting the underlying disease biology with the daily experience of hyperphagia, these expert perspectives can help inform how best to assess and manage the symptoms and thus support continued progress in care for the PWS community."

The manuscript brings together perspectives from global experts in endocrinology, psychiatry, neuroscience, obesity, genetics, and patient advocacy to advance understanding of hyperphagia in PWS and inform future research and clinical practice. It describes how disruptions in the hypothalamus neural pathways that regulate hunger, fullness, and food-related reward can interact with autonomic dysfunction and environmental factors to shape the varied experience of hyperphagia and its broader effects on individuals and families. Perspectives also outline considerations for assessment and management of hyperphagia and underscore the need for care that integrates biological, behavioral, and environmental approaches across childhood, adolescence, and adulthood. While environmental controls, such as locking cabinets and refrigerators or avoiding social situations where food is present, remain important for managing food access, the publication highlights the need for a more comprehensive approach that addresses the underlying complexity of hyperphagia.

Key themes from the publication include:

- **Hyperphagia is multidimensional.** Experts described hyperphagia as arising from multiple interconnected factors, including changes in how the brain regulates hunger, fullness, and food-related reward, as well as behavioral and environmental factors, with consequences extending beyond food intake alone.
- **Assessment must capture the full experience.** Experts noted that individuals living in highly structured environments may show relatively few observable food-seeking behaviors despite experiencing persistent thoughts about food and a strong drive to eat. As a result, existing assessment tools may underestimate disease severity, as well as success, in some patients. Evaluation should be multidimensional and consider food-related thoughts and behaviors, clinical observations, environmental context, and caregiver perspectives.
- **Adolescence and transition to adult care are particularly vulnerable periods.** Loss of structured support systems, fragmented care, increased independence, and the associated increase in uncertainty, combined with family exhaustion, may contribute to worsening symptoms and increased health risks. Continued expert support is essential to reduce potential clinical complications and maintain quality of life.
- **Integrated management remains essential.** Food security and controlled food environments continue to be foundational therapeutic intervention rather than environment management alone. Experts emphasized the need to combine biological,

behavioral, and environmental approaches within multidisciplinary care, with particular attention to vulnerabilities during transition periods.

- **Pharmacologic treatment can complement foundational support.** Medication may lessen the burden of hyperphagia on individuals and caregivers by providing more flexibility in constant management. In this context, [VYKAT® XR](#) (diazoxide choline) extended-release tablets represent a significant advancement as the first FDA-approved treatment of hyperphagia in PWS. Pharmacological therapy is an important component of hyperphagia management and should be considered in multidisciplinary care.

"Hyperphagia in Prader-Willi syndrome extends far beyond hunger or food intake. It affects behavior, autonomy, and daily life for individuals living with PWS and their families," said Maithé Tauber, M.D., Professor Emeritus of Pediatrics at Université Toulouse III and lead author of the publication. "Improving care will rely on a truly integrated approach, one that deepens our understanding of the underlying biology, reflects the experience of individuals and their families, and actively engages healthcare teams within well-coordinated systems of care that support patients from birth throughout their lives."

Scientific and clinical experts also identified additional priorities for research and clinical care in PWS, including the development of validated outcome measures for clinical trials, identification of biomarkers, and creation of care models that more closely connect mechanistic insights with real-world clinical practice.

About Prader-Willi Syndrome

Prader-Willi syndrome (PWS) is a rare genetic neurodevelopmental disorder caused by the loss of expression of specific genes on the paternally inherited copy of chromosome 15. The Prader-Willi Syndrome Association USA estimates that PWS occurs in one in every 15,000 live births. The defining symptom of PWS is hyperphagia, a chronic and life-threatening condition neurologically driven by an intense, persistent sensation of hunger and characterized by food preoccupations, an extreme drive to consume food, food-related behavior problems, and a lack of normal satiety, which can severely diminish the quality of life for individuals with PWS and their families.

Hyperphagia can lead to significant mortality (e.g., stomach rupture, choking, accidental death due to food-seeking behavior) and longer-term co-morbidities such as diabetes, obesity, and cardiovascular disease.

About VYKAT® XR (diazoxide choline) Extended-Release Tablets

VYKAT XR was approved by the U.S. Food and Drug Administration (FDA) on March 26, 2025, and is now commercially available to U.S. patients.

VYKAT XR is indicated for the treatment of hyperphagia in adults and pediatric patients 4 years of age and older with Prader-Willi syndrome (PWS).

IMPORTANT SAFETY INFORMATION

Contraindications

Use of VYKAT XR is contraindicated in patients who have a known hypersensitivity to diazoxide, other components of VYKAT XR, or to thiazides.

Warnings and Precautions

Hyperglycemia

Hyperglycemia, including diabetic ketoacidosis, has been reported. Before initiating VYKAT XR, test fasting plasma glucose (FPG) and HbA1c; optimize blood glucose in patients who have hyperglycemia. During treatment, regularly monitor fasting glucose (FPG or fasting blood glucose) and HbA1c. Monitor fasting glucose more frequently during the first few weeks of treatment in patients with risk factors for hyperglycemia.

Risk of Fluid Overload

Edema, including severe reactions associated with fluid overload, has been reported. Monitor for signs or symptoms of edema or fluid overload. VYKAT XR has not been studied in patients with compromised cardiac reserve and should be used with caution in these patients.

Adverse Reactions

The most common adverse reactions (incidence $\geq 10\%$ and at least 2% greater than placebo) included hypertrichosis, edema, hyperglycemia, and rash.

Please see the full [Prescribing Information, including Medication Guide](#).

About Neurocrine Biosciences, Inc.

Neurocrine Biosciences is a leading biopharmaceutical company with a simple purpose: to relieve suffering for people with great needs. We are dedicated to discovering, developing, and commercializing life-changing treatments for patients with under-addressed neurological, psychiatric, endocrine, and immunological disorders. The company's diverse portfolio includes FDA-approved treatments for tardive dyskinesia, chorea associated with Huntington's disease, classic congenital adrenal hyperplasia, hyperphagia in Prader-Willi syndrome, endometriosis*, and uterine fibroids*, as well as a robust pipeline including multiple compounds in mid- to late-phase clinical development across our core therapeutic areas. For more than three decades,

we have applied our unique insight into neuroscience and the interconnections between brain and body systems to treat complex conditions. We relentlessly pursue medicines to ease the burden of debilitating diseases and disorders, because you deserve brave science. For more information, visit neurocrine.com, and follow the company on [LinkedIn](#), [X](#), [Facebook](#), and [YouTube](#). (**in collaboration with AbbVie*)

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Forward-Looking Statements

In addition to historical facts, this press release contains forward-looking statements that involve a number of risks and uncertainties. These statements include, but are not limited to, statements regarding the potential benefits to be derived from VYKAT XR for the treatment of Prader-Willi syndrome (PWS); the value and benefits VYKAT XR brings to patients with PWS; and the potential for the expert perspectives described in the publication to inform future research, clinical practice, and the assessment and management of hyperphagia in PWS. Factors that could cause actual results to differ materially from those stated or implied in the forward-looking statements include, but are not limited to, the following: risks and uncertainties regarding whether the expert perspectives described in the publication will inform future research, clinical practice, or the assessment and management of hyperphagia in PWS; risks and uncertainties associated with our business and finances in general, as well as risks and uncertainties associated with the commercialization of VYKAT XR, including the extent to which patients and physicians accept and adopt VYKAT XR; whether VYKAT XR receives adequate coverage and reimbursement from third-party payors; risks and uncertainties relating to competitive products and technological changes that may limit demand for VYKAT XR; risks associated with dependence on third parties for development and manufacturing activities related to VYKAT XR, and risks associated with managing these third parties; risks that additional regulatory submissions for VYKAT XR may not occur or be submitted in a timely manner; risks that the FDA or other regulatory authorities may make adverse decisions regarding VYKAT XR; risks that post-approval commitments or requirements for VYKAT XR may be delayed; risks that VYKAT XR may be precluded from commercialization by the proprietary or regulatory rights of third parties, or have unintended side effects, adverse reactions or incidents of misuse; and other risks described in the Company's periodic reports filed with the Securities and Exchange Commission, including without limitation the Company's quarterly report on Form 10-Q for the quarter ended June 30, 2026. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof except as required by law.

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