



Neurocrine Biosciences to Present Research Advancing Understanding of Prader-Willi Syndrome and Real-World Experience with VYKAT® XR (diazoxide choline) at Foundation for Prader-Willi Research Symposium & Family Conference

September 30, 2026

- PWS-VISTA observational study will build longitudinal real-world evidence on VYKAT XR use for hyperphagia in Prader-Willi syndrome in routine clinical practice
- BRAVE-PWS observational study will capture caregiver-reported information to better understand the experience and burden of caring for someone with Prader-Willi syndrome
- Integrated analysis will further evaluate adverse events of special interest — including fluid retention, hyperglycemia, hirsutism and hypertrichosis — during VYKAT XR treatment

SAN DIEGO, Sept. 30, 2026 /PRNewswire/ -- [Neurocrine Biosciences, Inc.](#) (Nasdaq: NBIX) today announced research advancing understanding of Prader-Willi syndrome and expanding clinical and real-world evidence for [VYKAT® XR](#) (diazoxide choline) extended-release tablets will be presented at the Foundation for Prader-Willi Research (FPWR) 2026 Annual Research Symposium & Family Conference, taking place Oct. 7-10 in Philadelphia.



The research reflects Neurocrine's continued commitment to ongoing education of Prader-Willi syndrome (PWS) and VYKAT XR — the first and only FDA-approved treatment for hyperphagia in people aged 4 years and older living with PWS — through clinical and real-world evidence. It includes:

- PWS-VISTA, an ongoing observational study designed to build longitudinal real-world evidence on VYKAT XR use in routine clinical practice, including long-term safety and treatment outcomes. Neurocrine will present baseline demographic and clinical characteristics of PWS patients initiating VYKAT XR.
- BRAVE-PWS, designed with caregiver and clinician input, is an ongoing observational study intended to capture caregiver-reported information to better understand the experience and burden of caring for someone with PWS.
- Additional research will examine the timing and occurrence of adverse events of special interest during VYKAT XR treatment, including fluid retention, hyperglycemia, hirsutism and hypertrichosis.

"Prader-Willi syndrome is a complex, rare condition that profoundly affects individuals and their families, and continued research is essential to deepening our understanding of PWS and the needs of the community," said Eiry W. Roberts, M.D., Chief Medical Officer, Neurocrine Biosciences. "The breadth of research we are presenting at the FPWR Research Symposium & Family Conference reflects our long-term commitment to individuals living with PWS, caregivers, healthcare providers, researchers and the broader PWS community. These findings can help inform how we apply our scientific and clinical expertise to areas of great unmet need."

Neurocrine will share the following oral presentation and posters at the FPWR 2026 Annual Research Symposium & Family Conference. All times are in Eastern time.

Title: Real-World Assessment of GLP-1 Receptor Agonist Utilization and Risk of Gastrointestinal Complications in Prader-Willi Syndrome

Presenting Author: Shawn McCandless, M.D.

Date/Time: Oct. 8, from 9:30 to 9:50 a.m.

Title: Real-World Demographics and Clinical Characteristics of Patients with Prader-Willi Syndrome Initiating VYKAT XR (Diazoxide Choline) Extended-Release Tablets: Baseline Results from the PWS-VISTA Observational Study (**Poster Presentation #6**)

Presenting Author: Julie Perry, M.D.

Date/Time: Oct. 7, from 5 to 7 p.m.

Title: BRAVE-PWS: Burden and Real-World Assessment of Caregiver Voice and Experiences in Prader-Willi Syndrome—Study Design of an Observational, App-Based Health-Tracking Cohort Study (**Poster Presentation #8**)

Presenting Author: Christopher Joshi, M.D., MPH

Date/Time: Oct. 7, from 5 to 7 p.m.

Title: Integrated Exposure-Adjusted Analysis of Adverse Events of Special Interest During Diazoxide Choline Treatment for Prader-Willi Syndrome (**Poster Presentation #15**)

Presenting Author: Sheri Leung, Pharm.D.

Date/Time: Oct. 7, from 5 to 7 p.m.

Title: Mortality Among Patients with Prader-Willi Syndrome (MAP-PWS): An Analysis of Healthcare Utilization in the Year Prior to Death Within a Single U.S. Payer (**Poster Presentation #16**)

Presenting Author: Raj Gandhi, Pharm.D., MBA

Date/Time: Oct. 7, from 5 to 7 p.m.

Title: Long-Term Reductions of Hyperphagia with Diazoxide Choline Extended-Release (DCCR) in Participants with Prader-Willi Syndrome (**Poster Presentation #12**)

Presenting Author: Evelien F. Gevers, M.D., Ph.D., FRCPCH

Date/Time: Oct. 7, from 5 to 7 p.m.

Title: Efficacy and Safety of Resuming Diazoxide Choline Extended-Release (DCCR) after 16-week Randomized Withdrawal in Prader-Willi Syndrome (PWS) (Study C614) (**Poster Presentation #14**)

Presenting Author: Evelien F. Gevers, M.D., Ph.D., FRCPCH

Date/Time: Oct. 7, from 5 to 7 p.m.

Title: Impact of Long-Term Diazoxide Choline Extended-Release (DCCR) Treatment and the Prader-Willi Syndrome Profile Questionnaire (PWSP) (**Poster Presentation #13**)

Presenting Author: Evelien F. Gevers, M.D., Ph.D., FRCPCH

Date/Time: Oct. 7, from 5 to 7 p.m.

About Prader-Willi Syndrome

Prader-Willi syndrome (PWS) is a rare genetic neurodevelopmental disorder caused by an abnormality of chromosome 15 gene expression. 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](https://www.neurocrine.com), and follow the company on [LinkedIn](#), [X](#), [Facebook](#) and [YouTube](#). (*in collaboration with AbbVie)

The NEUROCRINE BIOSCIENCES Logo, NEUROCRINE and YOU DESERVE BRAVE SCIENCE are registered trademarks of Neurocrine Biosciences, Inc. SOLENO and VYKAT are registered trademarks of Soleno Therapeutics, Inc.

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 Company's plans to advance understanding of PWS and expand clinical and real-world evidence regarding VYKAT XR through the studies and analyses described in this press release. 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 as to whether the Company can successfully conduct the studies and analyses described in this press release and whether such research will advance understanding of PWS and expand clinical and real-world evidence regarding VYKAT XR; 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.

© 2026 Neurocrine Biosciences, Inc. All Rights Reserved. CAP-NBI-US-0269 09/2026

 View original content to download multimedia: <https://www.prnewswire.com/news-releases/neurocrine-biosciences-to-present-research-advancing-understanding-of-prader-will-syndrome-and-real-world-experience-with-vykat-xr-diazoxide-choline-at-foundation-for-prader-will-research-symposium--family-conference-302893567.html>

SOURCE Neurocrine Biosciences, Inc.

Neurocrine Biosciences, Inc., Media: Tony Jewell, 1-858-354-7865, media@neurocrine.com; Investors: Todd Tushla, 1-858-617-7143, ir@neurocrine.com