# Neurocrine Biosciences to Present New Two-Year CRENESSITY® (crinecerfont) Data on Key Clinical and Patient-Reported Outcome Measures at ENDO 2026

June 3, 2026

- Analyses span adult and pediatric populations with classic congenital adrenal hyperplasia (CAH) and reflect longer-term clinical outcomes relevant to disease management across lifespan and care continuum
- New data demonstrate the effect of CRENESSITY on long-term androgen control and glucocorticoid dose reduction and associated clinical outcomes
- Data from cross-sectional surveys highlight patient- and caregiver-reported quality of life improvements
- Case series highlights use of CRENESSITY in patients with classic CAH due to 11β-hydroxylase deficiency
- Additional ENDO 2026 presentations include VYKAT™ XR (diazoxide choline) extended-release tablets data in hyperphagia associated with Prader-Willi syndrome (PWS), including late-breaking long-term extension findings following randomized withdrawal, and data evaluating sustained improvements in hyperphagia and behavioral symptoms through three years

SAN DIEGO, June 3, 2026 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced it will present multiple new analyses of key clinical and patient-reported outcomes up to two years of treatment with CRENESSITY® (crinecerfont) in adult and pediatric populations with classic congenital adrenal hyperplasia. These data will be presented at the Endocrine Society's annual meeting, ENDO 2026, taking place from June 13-16, in Chicago.

The presentations will highlight the breadth of data generated from the CAHtalyst® clinical program's long-term extension studies, reflecting Neurocrine's continued focus on advancing ongoing disease management in classic congenital adrenal hyperplasia (CAH). The data build on prior scientific presentations at recent medical meetings, including the American Association of Clinical Endocrinology 2026 Annual Meeting and the Pediatric Endocrine Society 2026 Annual Meeting. Together, these analyses extend the evaluation of longer-term clinical and patient-relevant outcomes associated with sustained androgen control and reduced glucocorticoid (GC) exposure.

"At ENDO 2026, we look forward to presenting compelling two-year treatment outcomes in patients with classic CAH treated with CRENESSITY," said Sanjay Keswani, M.D., Chief Medical Officer, Neurocrine Biosciences. "These data support the growing body of evidence around the meaningful long-term benefits of improved androgen control together with reduced exposure to high-dose glucocorticoids. This is the promise of treatment with CRENESSITY, which, together with lower-dose glucocorticoids, is rapidly becoming the new standard of care in classic CAH."

Across multiple adult and pediatric analyses presented at ENDO 2026, Neurocrine will share a range of endpoints intended to further characterize longer-term outcomes relevant to patients and clinicians, including measures related to metabolic health, bone health, growth and development and patient-reported quality of life.

In addition, Neurocrine will present a case series on the use of CRENESSITY in patients with classic CAH due to 11β-hydroxylase deficiency, the second most common type of classic CAH after 21-hydroxylase deficiency, representing approximately 5% of all cases.

Neurocrine will share the following poster and oral presentations at ENDO 2026. All times are Central Time:

CAHtalyst Adult Study Two-Year Results

**Title:** Weight-Related Outcomes and Insulin Resistance in Adults with Classic Congenital Adrenal Hyperplasia: 2-Year Results from the CAHtalyst Adult Study **(Oral Presentation #ORF32-07)**
**Authors:** Oksana Hamidi, D.O., et al
**Date/Time:** June 14 from 2:55-3:10 PM

**Title:** Adults with Classic Congenital Adrenal Hyperplasia Taking Crinecerfont Demonstrated Sustained Decreases in Glucocorticoid Doses: 2-Year Results from the CAHtalyst Adult Study **(Poster Presentation #SUN-458)**
**Authors:** Irina Bancos, M.D., et al
**Date/Time:** June 14 from 12:00-1:30 PM

**Title:** A Cross-sectional Survey on Quality of Life of Adults with Classic Congenital Adrenal Hyperplasia in the United States Participating in CAHtalyst Adult Open-Label Extension Study **(Poster Presentation #SUN-467)**
**Authors:** Sonal Vaid, M.D., et al
**Date/Time:** June 14 from 12:00-1:30 PM

**Title:** Bone Outcomes in Adults with Classic Congenital Adrenal Hyperplasia Treated with Crinecerfont for Up to 2 Years in CAHtalyst Adult Study **(Poster Presentation #SUN-468)**
**Authors:** Maria Vogiatzi, M.D., et al
**Date/Time:** June 14 from 12:00-1:30 PM

CAHtalyst Pediatric Study Two-Year Results

**Title:** Characterization of Children and Adolescents with Classic Congenital Adrenal Hyperplasia Who Had Slowed Bone Age Progression and Improved Height Prediction with Crinecerfont **(Oral Presentation #ORF32-05)**
**Authors:** Maria Vogiatzi, M.D., et al
**Date/Time:** June 14 from 2:25-2:40 PM

**Title:** Long-term Crinecerfont Treatment Reduced ACTH and 17-Hydroxyprogesterone — Clinical Outcomes in Children and Adolescents with Classic Congenital Adrenal Hyperplasia: 2-Year Results from CAHtalyst Pediatric **(Poster Presentation #SAT-465)**
**Authors:** Natalie Nokoff, M.D., et al
**Date/Time:** June 13 from 12:15-1:45 PM

**Title:** Long-term Crinecerfont Enables Sustained Decreases in Glucocorticoid Doses — Clinical Outcomes in Children and Adolescents with Classic Congenital Adrenal Hyperplasia: 2-Year Results from CAHtalyst Pediatric **(Poster Presentation #SUN-465)**
**Authors:** Kyriakie Sarafoglou, M.D., et al
**Date/Time:** June 14 from 12:00-1:30 PM

Additional Presentations

*Classic CAH:*

**Title:** Long-Term Risk of Cardiometabolic Comorbidities Associated with Glucocorticoid Exposure and Androgen Control in Classic Congenital Adrenal Hyperplasia: A Cox Proportional Hazards Analysis from the CAHtalog Registry **("New Therapies and Perspectives for Congenital Adrenal Hyperplasia and Adrenal Insufficiency" Rapid Fire Presentation #ORF32-02 and Poster Presentation #MON-495)**
**Authors:** Oksana Lekarev, D.O., et al
**Date/Time:** June 14 from 1:50-1:55 PM (Rapid Fire) and June 15 from 9:00-2:00 PM (Poster)

**Title:** Crinecerfont Treatment of Classic Congenital Adrenal Hyperplasia Due to 11β-Hydroxylase Deficiency: A Case Series **(Poster Presentation #SAT-466)**
**Authors:** Kyriakie Sarafoglou, M.D., et al
**Date/Time:** June 13 from 12:15-1:45 PM

**Title:** A Modified Delphi Panel of U.S. Endocrinologists to Align on Minimum Clinically Important Difference in Glucocorticoid Dose and Other Key Considerations in Classic Congenital Adrenal Hyperplasia **(Poster Presentation #SAT-459)**
**Authors:** Ahmed Khattab, M.D., et al
**Date/Time:** June 13 from 12:15-1:45 PM

*PWS:*

**Title:** *(Late-breaker)* Efficacy and Safety of Resuming Diazoxide Choline Extended-Release after 16-Week Randomized Withdrawal in Prader-Willi Syndrome (Study C614) **(Poster Presentation #SUN-689)**
**Authors:** Jennifer L. Miller, M.D., et al
**Date/Time:** June 14 from 12:00-1:30 PM

**Title:** Long-Term Reductions of Hyperphagia with Diazoxide Choline Extended-Release in Participants with Prader-Willi Syndrome **(Poster Presentation #SUN-688)**
**Authors:** Evelien F. Gevers, M.D., Ph.D., et al
**Date/Time:** June 14 from 12:00-1:30 PM

**Title:** Impact of Long-Term Diazoxide Choline Extended-Release Treatment and the Prader-Willi Syndrome Profile Questionnaire **("Hypothalamic and Genetic Disorders of Energy Balance" Rapid Fire Presentation #ORF44-02 and Poster**

**Presentation #MON-690)**
**Authors:** Evelien F. Gevers, M.D., Ph.D., et al
**Date/Time:** June 15 from 9:35-9:40 AM (Rapid Fire) and June 15 from 9:00-2:00 PM (Poster)

**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 #MON-863)**
**Authors:** Isabella Niu, M.D., et al
**Date/Time:** June 15 from 12:00-1:30 PM

**About Congenital Adrenal Hyperplasia**
Congenital adrenal hyperplasia (CAH) is a rare genetic condition that results in an enzyme deficiency that alters the production of adrenal steroid hormones, such as cortisol, aldosterone and adrenal androgens. Severe enzyme deficiency leads to an inability of the adrenal glands to produce enough cortisol and, in approximately 75% of cases, aldosterone. Because individuals with CAH are typically still able to produce androgens, the unused precursors that would normally be used to make cortisol instead result in the production of excess amounts of androgens. If left untreated, CAH can result in adrenal crisis and even death.

Exogenous glucocorticoids (GCs) are necessary to correct the endogenous cortisol deficiency, but historically, doses higher than those needed for cortisol replacement (supraphysiologic) have been used to lower the elevated levels of adrenocorticotropic hormone (ACTH) and adrenal androgens. However, GC treatment at supraphysiologic doses has been associated with serious and significant complications of steroid excess, including metabolic issues such as weight gain and diabetes, cardiovascular disease and osteoporosis. Additionally, long-term treatment with supraphysiologic GCs may have psychological and cognitive impacts, such as changes in mood and memory. Adrenal androgen excess has been associated with abnormal bone growth and development in pediatric patients, female health problems such as excess facial hair growth and menstrual irregularities, in addition to cardiometabolic and fertility issues in both sexes. The symptoms of high ACTH may include testicular adrenal rest tumors (TARTs).

**About CRENESSITY® (crinecerfont)**
CRENESSITY is a potent and selective oral corticotropin-releasing factor type 1 receptor ($CRF_1$) antagonist that reduces and controls excess adrenocorticotropic hormone (ACTH) and adrenal androgens through a non-glucocorticoid (GC) mechanism for the treatment of classic congenital adrenal hyperplasia (CAH). Antagonism of $CRF_1$ receptors in the pituitary has been shown to decrease ACTH levels, which in turn decreases the production of adrenal androgens and potentially the symptoms associated with CAH. The robust clinical study data demonstrate that lowering adrenal androgen levels with CRENESSITY enables lower, more physiologic dosing of GCs to replace missing cortisol.

CRENESSITY comes in capsules and an oral solution. For adults 18 years of age and older, the recommended dosage is 100 mg twice daily taken orally with a meal. For pediatric patients four to 17 years of age weighing less than 55 kg (121 lbs), the recommended dosage is based on body weight and is administered twice daily, taken orally with a meal. For pediatric patients weighing more than 55 kg (121 lbs), the recommended dosage is 100 mg twice daily taken orally with a meal. Healthcare providers can work with patients to determine the appropriate formulation for use depending on patient needs. Patients receiving CRENESSITY should continue GC therapy for cortisol replacement.

**About the CAHtalyst® Studies**
The Phase 3 CAHtalyst global registrational studies were designed to evaluate the safety, efficacy and tolerability of CRENESSITY® (crinecerfont) in children and adults with classic congenital adrenal hyperplasia (CAH) due to 21-hydroxylase deficiency. The CAHtalyst studies were the largest-ever clinical trial program in classic CAH, including 285 pediatric and adult patients.

The CAHtalyst Pediatric study included 103 pediatric patients four to 17 years of age. The study tested two questions. The first question evaluated whether four weeks of CRENESSITY treatment could improve androgen control. The second question evaluated whether an additional 24 weeks of CRENESSITY treatment enabled customized glucocorticoid (GC) down-titration while androstenedione levels were maintained or improved.

The CAHtalyst Adult study included 182 adult patients 18 to 58 years of age. Similarly, the first question of the study evaluated whether four weeks of CRENESSITY treatment could improve androgen control, and the second question evaluated whether an additional 20 weeks of CRENESSITY treatment enabled GC reduction to physiologic range while androstenedione levels were maintained or improved.

Data from the CAHtalyst Phase 3 studies supported approval of CRENESSITY by the U.S. Food and Drug Administration in December 2024. The open-label extension treatment portions of both studies are ongoing.

**Important Information**

**Approved Uses**
CRENESSITY® (crinecerfont) is a prescription medicine used together with glucocorticoids (steroids) to control androgen (testosterone-like hormone) levels in adults and children 4 years of age and older with classic congenital adrenal hyperplasia (CAH).

**IMPORTANT SAFETY INFORMATION**

**Do not take CRENESSITY if you:**

Are allergic to crinecerfont, or any of the ingredients in CRENESSITY.

**CRENESSITY may cause serious side effects, including:**

**Allergic reactions.** Symptoms of an allergic reaction include tightness of the throat, trouble breathing or swallowing, swelling of the lips, tongue, or face, and rash. If you have an allergic reaction to CRENESSITY, get emergency medical help right away and stop taking CRENESSITY.

**Risk of Sudden Adrenal Insufficiency or Adrenal Crisis with Too Little Glucocorticoid (Steroid) Medicine.** Sudden adrenal insufficiency or adrenal crisis can happen in people with congenital adrenal hyperplasia who are not taking enough glucocorticoid (steroid) medicine. You should continue taking your glucocorticoid (steroid) medicine during treatment with CRENESSITY. Certain conditions such as infection, severe injury, or shock may increase your risk for sudden adrenal insufficiency or adrenal crisis. Tell your healthcare provider if you get a severe injury, infection, illness, or have planned surgery during treatment. Your healthcare provider may need to change your dose of glucocorticoid (steroid) medicine.

**Before taking CRENESSITY, tell your healthcare provider about all of your medical conditions, including if you:** are pregnant or plan to become pregnant, or are breastfeeding or plan to breastfeed.

**Tell your healthcare provider about all the medicines you take,** including prescription and over-the-counter medicines, vitamins and herbal supplements.

**The most common side effects of CRENESSITY in adults include** tiredness, headache, dizziness, joint pain, back pain, decreased appetite, and muscle pain.

**The most common side effects of CRENESSITY in children include** headache, stomach pain, tiredness, nasal congestion, and nosebleeds.

These are not all the possible side effects of CRENESSITY. Call your healthcare provider for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit MedWatch at www.fda.gov/medwatch or call 1-800-FDA-1088.

**Dosage Forms and Strengths:** CRENESSITY is available in 50 mg and 100 mg capsules, and as an oral solution of 50 mg/mL.

Please see full **Prescribing Information**.

**About PWS**
Prader-Willi syndrome (PWS) is a rare genetic neurodevelopmental disorder caused by an abnormality in gene expression on 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 characterized by an intense persistent sensation of hunger accompanied 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 comorbidities such as diabetes, obesity, and cardiovascular disease.

**About VYKAT™ XR**
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).

**INDICATION**
VYKAT XR (diazoxide choline) extended-release tablets 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 ≥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 patients with 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)*



**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 CRENESSITY for the treatment of classic congenital adrenal hyperplasia (CAH) and VYKAT XR for the treatment of Prader-Willi syndrome (PWS); the value and benefits CRENESSITY brings to patients with CAH, including its potential to support long-term hormone control and glucocorticoid dose reduction; the value and benefits VYKAT XR brings to patients with PWS, including its potential to support sustained improvements in hyperphagia and behavioral symptoms; the ability of Neurocrine Biosciences to ensure patients have access to CRENESSITY and VYKAT XR; and whether the results from our clinical trials and other data analyses described in this press release are indicative of real-world results. 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 data described in this press release will be replicated in additional studies or will be predictive of efficacy or other clinical outcomes in subsequent clinical studies or real-world use of CRENESSITY or VYKAT XR; risks and uncertainties associated with Neurocrine Biosciences' business and finances in general, as well as risks and uncertainties associated with the commercialization of CRENESSITY and VYKAT XR, including the extent to which patients and physicians accept and adopt CRENESSITY and VYKAT XR; whether CRENESSITY and VYKAT XR receives adequate reimbursement from third-party payors; risks and uncertainties relating to competitive products and technological changes that may limit demand for CRENESSITY or VYKAT XR; risks associated with the Company's dependence on third parties for development and manufacturing activities related to CRENESSITY and VYKAT XR, and the ability of the Company to manage these third parties; risks that additional regulatory submissions for CRENESSITY or 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 CRENESSITY or VYKAT XR; risks that post-approval commitments or requirements for CRENESSITY or VYKAT XR may be delayed; risks that CRENESSITY or 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 March 31, 2026. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof other than required by law.



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SOURCE Neurocrine Biosciences, Inc.

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