Neurocrine Biosciences Presents First Retrospective Case Series of CRENESSITY® (crinecerfont) in Patients with Classic Congenital Adrenal Hyperplasia Due to 11β-Hydroxylase Deficiency at ENDO 2026

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- Androstenedione and other adrenal hormone levels in 11β-hydroxylase-deficient patients improved substantially after initiation of CRENESSITY, with >90% median reductions in 11-deoxycortisol and 11-deoxycorticosterone
- Nearly all patients (14/15) reduced their total glucocorticoid dose with CRENESSITY, and 2 of 5 patients on antihypertensive medications reduced or discontinued these drugs
- Findings provide initial clinical insights in patients with classic congenital adrenal hyperplasia due to 11β-hydroxylase deficiency, a rare subtype not previously studied in clinical trials of CRENESSITY

SAN DIEGO, June 15, 2026 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced clinical findings from the first retrospective case series in pediatric and adult patients with classic congenital adrenal hyperplasia (CAH) due to 11β-hydroxylase deficiency. This subtype was not previously studied in clinical trials of CRENESSITY® (crinecerfont) and is the second most common form of classic CAH after 21-hydroxylase deficiency, accounting for approximately 5% of cases. The findings were presented at the Endocrine Society's annual meeting, ENDO 2026, in Chicago.

Like other forms of CAH, 11β-hydroxylase deficiency (11β-OHD) is characterized by cortisol deficiency and excess adrenal androgens. However, 11β-OHD is uniquely associated with the accumulation of the adrenal steroid precursors 11-deoxycortisol (11-dF) and 11-deoxycorticosterone (DOC). These hormonal imbalances can contribute to distinct clinical features, including hypertension and other long-term complications. In this retrospective case series, reductions in androstenedione (A4) were observed across all patients with elevated baseline levels following initiation of CRENESSITY. Steroid precursors also decreased, with values reaching within normal ranges, and blood pressure improved in adult patients.

"Patients with 11β-hydroxylase deficiency represent a complex subgroup within classic congenital adrenal hyperplasia, with limited data available to guide treatment decisions for these individuals," said Sanjay Keswani, M.D., Chief Medical Officer, Neurocrine Biosciences. "This first case series provides preliminary clinical insights on the use of CRENESSITY to target ACTH and potentially improve hormonal control in patients with 11β-hydroxylase deficiency, highlighting our ongoing commitment to advancing care for the entire classic CAH community."

This retrospective case series outlines clinical responses to CRENESSITY treatment in 15 pediatric (n=11) and adult (n=4) patients with classic CAH due to 11β-OHD. Across all patients with elevated A4 and/or adrenal steroid precursors at baseline, initiation of CRENESSITY was associated with reduction of levels.

- A4 and precursor normalization were observed as early as one month following treatment initiation, including in patients whose hormone excess had persisted despite prior supraphysiologic glucocorticoid (GC) therapy.
    - Among patients with elevated hormones at baseline, median decreases of -92% (DOC, n=5), -95% (11-dF, n=7), and -65% (A4, n=3) were observed after CRENESSITY initiation.
- Among patients receiving antihypertensive treatment, two of five were able to reduce or discontinue these medications.
- Following the initiation of CRENESSITY, 14 of 15 patients were able to reduce their total GC dose.

"Patients with 11β-hydroxylase deficiency often experience both androgen excess and elevated adrenal steroid precursors that can contribute to hypertension and other complications, making disease management particularly challenging," said Kyriakie Sarafoglou, M.D., Professor, Department of Pediatrics and Department of Experimental and Clinical Pharmacology, Divisions of Endocrinology and Genetics & Metabolism, University of Minnesota. "Given the rarity of this condition, data from 15 patients gives clinicians meaningful insight into the potential role of CRENESSITY in managing this challenging form of classic congenital adrenal hyperplasia."

This case series provides early, foundational evidence supporting the efficacy and safety of CRENESSITY treatment in patients with classic CAH due to 11β-OHD. Although CRENESSITY is approved as an adjunctive treatment to GCs for patients with classic CAH regardless of enzyme deficiency, there is limited evidence on its use in this rare subtype. These findings support further exploration of CRENESSITY in this classic CAH subtype and reinforce Neurocrine's commitment to supporting patients across the full spectrum of classic CAH and other rare endocrine diseases.

Presentations at the ENDO 2026 annual meeting included:

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

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

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

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

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

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

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

Additional Presentations

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

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

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

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 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); the value and benefits CRENESSITY brings to patients with CAH, including its potential to target adrenocorticotropic hormone (ACTH) and improve hormonal control in patients with CAH due to 11β-hydroxylase deficiency; the ability of Neurocrine Biosciences to ensure patients have access to CRENESSITY; and whether the results from our clinical trials of CRENESSITY 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; risks and uncertainties associated with Neurocrine Biosciences' business and finances in general, as well as risks and uncertainties associated with the commercialization of CRENESSITY, including the extent to which patients and physicians accept and adopt CRENESSITY; whether CRENESSITY receives adequate reimbursement from third-party payors; risks and uncertainties relating to competitive products and technological changes that may limit demand for CRENESSITY; risks associated with the Company's dependence on third parties for development and manufacturing activities related to CRENESSITY, and the ability of the Company to manage these third parties; risks that additional regulatory submissions for CRENESSITY may not occur or be submitted in a timely manner; risks that the FDA or other regulatory authorities may make adverse decisions regarding CRENESSITY; risks that post-approval CRENESSITY commitments or requirements may be delayed; risks that CRENESSITY 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; risks and uncertainties relating to competitive products and technological changes that may limit demand for CRENESSITY; 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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