Neurocrine Biosciences Presents New Two-Year CRENESSITY® (crinecerfont) Data Showing Sustained Glucocorticoid Dose Reductions While Maintaining Androgen Control in Adults with Classic Congenital Adrenal Hyperplasia

April 22, 2026

- Approximately 70% of adult patients treated with CRENESSITY achieved and sustained physiologic-range glucocorticoid dosing at two years
- 75% of patients originally taking dexamethasone transitioned off this treatment, enabling a more physiologic glucocorticoid regimen without compromising androgen control
- Findings reinforce durable efficacy and a favorable long-term safety profile in largest interventional trial to date conducted in classic congenital adrenal hyperplasia

SAN DIEGO, April 22, 2026 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced the first presentation of new two-year data from the Phase 3 CAHtalyst® Adult study demonstrating sustained, substantial reductions in glucocorticoid (GC) doses in adults with classic congenital adrenal hyperplasia treated with CRENESSITY® (crinecerfont), with approximately 70% of patients achieving GC doses within the physiologic range. These data build upon previously reported one-year results and were presented at the American Association of Clinical Endocrinology 2026 Annual Meeting in Las Vegas.

Chronic exposure to supraphysiologic GC doses is associated with cardiometabolic comorbidities, bone density reductions, mental health issues and other long-term health risks that contribute to the cumulative treatment burden faced by patients over their lifetimes. Reducing high-dose, long-term GC exposure represents one of the most important goals in the management of classic congenital adrenal hyperplasia (CAH).

"For decades, the management of classic congenital adrenal hyperplasia has relied exclusively on supraphysiologic glucocorticoid dosing to control adrenal androgen and adrenocorticotropic hormone excess, exposing patients to significant cumulative long-term risks," said Sanjay Keswani, M.D., Chief Medical Officer, Neurocrine Biosciences. "These two-year findings demonstrated that CRENESSITY provided durable androgen control while enabling meaningful reductions in glucocorticoid exposure. Importantly, these reductions were sustained over time without new safety or tolerability concerns, supporting CRENESSITY as a long-term treatment option that advances the standard of care for people living with this complex condition."

At Month 24 of the study, 69% (103/149) of participants achieved a physiologic GC dose (≤11 mg/m^2/day hydrocortisone equivalents), with many participants eliminating nonphysiologic GC types. Of participants originally taking dexamethasone (n=20), 75% switched to a dexamethasone-free regimen, while 62% (37/60) of patients taking more than two doses of hydrocortisone per day were able to eliminate a dose outright. GC dose reductions and regimen changes were achieved without worsening androstenedione levels relative to baseline, indicating that lowering the GC dose was not achieved at the expense of androgen control.

Measure	Baseline	Month 18	Month 24
Mean daily GC dose (mg/m²/day HCe*), observed	17.6	10.6	10.6
Mean % change from baseline in GC dose, observed	—	-38 %	-38 %
Participants achieving physiologic GC dose (≤11mg/m²/day HCe*), n/N (%)	0/182 (0%)	114/161 (71%)	103/149 (69%)

*HCe denotes hydrocortisone equivalents.

Long-term treatment with CRENESSITY was generally well tolerated, with more than 80% study retention at two years and no new safety signals observed.

"Many years of supraphysiologic glucocorticoid exposure increase the risk for long-term health consequences, which include

obesity, diabetes, reduced bone density and psychosocial struggles," said Richard J. Auchus, M.D., Ph.D., Professor of Internal Medicine and Pharmacology, University of Michigan Medical School and Principal Investigator for the CAHtalyst Adult study. "These sequelae significantly impact quality of life and commonly develop with traditional CAH treatment regimens. The two-year findings provide important information on the durable benefit of treatment with CRENESSITY. Providers can confidently incorporate this additional knowledge to guide their management of adult patients with CAH into the future."

Neurocrine will be sharing additional two-year data across clinical endpoints and outcomes at upcoming medical meetings.

Additional presentation at the American Association of Clinical Endocrinology 2026 Annual Meeting includes:

- Cardiometabolic Outcomes Associated with Chronic Supraphysiologic Glucocorticoid Exposure and Crinecerfont Treatment: 1-Year Results from CAHtalyst Adult

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® 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 and developing life-changing treatments for patients with under-addressed neurological, endocrine, psychiatric 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, 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 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 enable patients to transition toward more physiologic glucocorticoid dosing; 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 annual report on Form 10-K for the year ended December 31, 2025. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof other than required by law.

View original content to download multimedia:https://www.prnewswire.com/news-releases/neurocrine-biosciences-presents-new-two-year-crenessity-crinecerfont-data-showing-sustained-glucocorticoid-dose-reductions-while-maintaining-androgen-control-in-adults-with-classic-congenital-adrenal-hyperplasia-302749451.html

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