



Neurocrine Biosciences Announces Publication on Traditional Glucocorticoid Treatment in Classic Congenital Adrenal Hyperplasia in Expert Review of Endocrinology & Metabolism

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- Narrative Review Assesses Current Treatment Challenges and the Evolving Classic Congenital Adrenal Hyperplasia (CAH) Therapeutic Landscape
- Non-Glucocorticoid (GC) Mechanisms for Treatment of CAH Could Enable Control of Excess ACTH and Androgens Without the Need for High-Dose GCs, Reducing Related Complications Over a Lifetime

SAN DIEGO, Jan. 8, 2025 /PRNewswire/ -- [Neurocrine Biosciences, Inc.](#) (Nasdaq: NBIX) today announced publication of a narrative review discussing the challenges of traditional treatment of classic congenital adrenal hyperplasia (CAH) with glucocorticoids (GCs) alone and the potential benefits of introducing novel non-GC mechanisms for treating the condition that may enable lower, more physiologic GC dosing. The publication appears in *Expert Review of Endocrinology & Metabolism*.



Treatment of classic CAH is a lifelong challenge that involves balancing the need to manage excess adrenocorticotropic hormone (ACTH) and androgens — historically achieved through high doses of GCs — while managing the risks associated with GC-related complications. The review, [Glucocorticoid Therapy in Classic Congenital Adrenal Hyperplasia: Traditional and New Treatment Paradigms](#), provides information for healthcare providers as they navigate the evolving classic CAH therapeutic landscape.

"The treatment paradigm for CAH involves continually monitoring and balancing ACTH and androgen levels and glucocorticoid-dosing to optimize treatment," said Irina Bancos, M.S., M.D., Professor of Medicine, Division of Endocrinology at the Mayo Clinic. "New CAH medications that control excess ACTH and adrenal androgens through a non-glucocorticoid mechanism allow for glucocorticoid dose reduction, with the cumulative reduction over time translating into a significant decrease in the risk for complications that can occur when higher doses are used over a patient's lifetime."

"Chronic exposure to high-dose glucocorticoid treatment can result in severe cardiovascular, metabolic and skeletal complications, as well as negatively impact the mental health and quality of life of patients," said Eiry W. Roberts, M.D., Chief Medical Officer, Neurocrine Biosciences. "Over a lifetime, even modest reductions in daily glucocorticoid doses can reduce the risk of these complications and lessen the overall burden of glucocorticoid exposure."

CAH presents a significant challenge for individuals living with the condition, their families and the healthcare providers who treat them. The condition leads to excess androgen production and has traditionally required high-dose GC treatment, both of which can have serious consequences. Until recently, treatment options have been limited, highlighting the unmet need for innovative new therapies for CAH.

The FDA recently approved [CRENESSITY™ \(crinecerfont\)](#) as an adjunctive treatment to GC replacement to control androgens in adult and pediatric patients four years of age and older with classic CAH. CRENESSITY, a potent and selective oral corticotropin-releasing factor type 1 receptor (CRF₁) antagonist, is the first and only classic CAH treatment that directly reduces excess ACTH and downstream adrenal androgen production, allowing for GC dose reduction.

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, which are essential for life. Approximately 95% of CAH cases are caused by variants of the *CYP21A2* gene that leads to deficiency of the enzyme 21-hydroxylase (21-OH). Severe deficiency of this enzyme leads to an inability of the adrenal glands to produce enough cortisol and, in approximately 75% of cases, aldosterone. Because individuals with CAH are 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 salt wasting, dehydration and even death.

Historically, exogenous glucocorticoids (GCs) have been used not only to correct the endogenous cortisol deficiency, but doses used are higher than cortisol replacement needed (supraphysiologic) to lower the levels of adrenocorticotropic hormone (ACTH) and adrenal androgens. However, GC treatment at high 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 high-dose GCs may have psychological and cognitive impact, 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, testicular rest tumors in males and fertility issues in both sexes.

About CRENESSITY™ (crinecerfont)

CRENESSITY™ is a potent and selective, oral corticotropin-releasing factor type 1 receptor (CRF₁) antagonist developed to reduce and control excess adrenocorticotropic hormone (ACTH) and adrenal androgens through a non-glucocorticoid (GC) mechanism for the treatment of classic congenital adrenal hyperplasia (CAH). Antagonism of CRF₁ 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. The capsule formulation is available in 50 mg and 100 mg doses. The oral solution is available as a 50 mg/mL strength formulation. For adults 18 years 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.

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 nose bleeds.

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 neuroscience-focused, 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, neuroendocrine and neuropsychiatric 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](https://www.neurocrine.com), and follow the company on [LinkedIn](#), [X \(formerly Twitter\)](#) and [Facebook](#). (**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 CRENESSITY for the treatment of classic congenital adrenal hyperplasia (CAH); the value and benefits CRENESSITY brings to patients with CAH; 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 associated with Neurocrine Biosciences' business and finances in general, as well as risks and uncertainties associated with the commercialization of CRENESSITY; whether CRENESSITY receives adequate reimbursement from third-party payors; the degree and pace of market uptake of CRENESSITY; 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 or other product candidates 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 September 30, 2024. 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