Neurocrine Biosciences Presents New Two-Year CRENESSITY® (crinecerfont) Data Demonstrating Improved Growth Measures in Pediatric Patients with Classic Congenital Adrenal Hyperplasia at ENDO 2026

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- Two-year data showed that pediatric patients with accelerated bone age at baseline experienced slowed bone age progression and improved predicted adult height with CRENESSITY
- Cross-sectional caregiver survey data showed universal satisfaction with CRENESSITY, willingness to recommend treatment and increased optimism about their child's future quality of life following treatment initiation

SAN DIEGO, June 15, 2026 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced new two-year data from the Phase 3 CAHtalyst® Pediatric study showing positive growth outcomes in children and adolescents with classic congenital adrenal hyperplasia treated with CRENESSITY® (crinecerfont). Patients with advanced bone age at baseline experienced slowed bone age progression and improved predicted adult height after two years of treatment. These data were presented at the Endocrine Society's annual meeting, ENDO 2026, in Chicago.

"Treatment decisions made during growth years can have lifelong implications for children and adolescents with classic congenital adrenal hyperplasia," said Sanjay Keswani, M.D., Chief Medical Officer, Neurocrine Biosciences. "Excess adrenal androgens can accelerate bone maturation and limit adult height. These two-year data suggest that sustained androgen control with CRENESSITY, combined with reduced glucocorticoid exposure, may help address abnormal growth in pediatric patients by slowing the advancement of bone age and narrowing the gap between bone age and chronological age."

Pediatric participants who completed the 28-week, double-blind, placebo-controlled period and the subsequent six-month, open-label period of the CAHtalyst Pediatric study continued treatment with CRENESSITY in an ongoing, open-label extension.

Slowed Bone Age Progression and Improved Predicted Adult Height

In the subset of growing pediatric patients (n=41), changes in bone age and predicted adult height were evaluated at Month 24. Bone age was assessed using bone age standard deviation score (SDS), a measure that compares skeletal maturity to age- and sex-matched norms.

Among those with advanced bone age at baseline (n=24), bone age SDS remained stable or improved in most patients with up to two years of CRENESSITY treatment. Mean bone age SDS decreased by 1.12, with a subset of patients (n=9) demonstrating reductions greater than two standard deviations. In this subset of patients, mean predicted adult height increased by 4.7 cm from baseline. Together the data suggest that sustained hormonal control and substantial glucocorticoid (GC) reductions enabled by CRENESSITY may positively influence growth during childhood and adolescence.

"Accelerated bone age is one of the most challenging complications we face when managing classic congenital adrenal hyperplasia in growing children because it can permanently affect adult height," said Maria G. Vogiatzi, M.D., Division of Endocrinology at Children's Hospital of Philadelphia, Principal Investigator for the CAHtalyst Pediatric study and lead author of the growth analysis. "Seeing stabilization and even improvement in bone age over two years in a subset of patients is encouraging and suggests meaningful progress in addressing a key driver of compromised growth during the critical development years."

Caregiver-reported Survey Data Reflects Satisfaction with CRENESSITY and Increased Optimism

To further explore caregiver perspectives and patient experience following long-term treatment with CRENESSITY, a cross-sectional, caregiver-reported survey of eligible U.S. participants from the CAHtalyst Pediatric open-label extension (n=29; preliminary analysis) was conducted at patients' latest site visits.

Among caregivers in the preliminary survey analysis, 100% (29/29) reported:

- Overall satisfaction with CRENESSITY.
- Likelihood to recommend CRENESSITY to others with classic congenital adrenal hyperplasia (CAH).
- Increased optimism about reducing risks of long-term impacts associated with high-dose steroid use, including 79% (23/29) who reported feeling less concern about steroid-associated weight gain.

Nearly all respondents (97%, 28/29) reported greater overall hope for their child's future living with classic CAH, with 93% (27/29) indicating increased optimism about reducing long-term impact of high levels of adrenocorticotropic hormone and androgens.

Across analyses, CRENESSITY was generally well tolerated through two years of treatment in pediatric patients, with no new safety signals observed during long-term follow-up.

These analyses build on previously presented results showing that CRENESSITY enabled substantial, sustained reductions in mean GC dose over two years without compromising androgen control. Reduced GC exposure was associated with sustained improvements in clinical outcomes linked to long-term, supraphysiologic GC use, including insulin resistance, weight and body mass index. These findings suggest a reduction in cardiometabolic risk in pediatric patients with classic CAH.

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 (CRF1) 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 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. 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 help address abnormal growth in pediatric patients with CAH by slowing bone age progression and improving predicted adult height in patients with advanced bone age at baseline; 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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