Neurocrine Biosciences Initiates Phase 2 Study of Crinecerfont in Pediatric Patients Under 4 Years Old with Classic Congenital Adrenal Hyperplasia

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SAN DIEGO, July 1, 2026 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced the initiation of its Phase 2 clinical study to assess the safety and tolerability of crinecerfont in children aged 3 months to under 4 years with classic congenital adrenal hyperplasia (CAH). Crinecerfont, marketed as CRENESSITY®, is approved in the United States as an adjunctive treatment to glucocorticoid replacement to control androgens in adult and pediatric patients 4 years of age and older with classic CAH.

"Infants and young children with classic CAH face significant health challenges and are often exposed to high doses of glucocorticoids during critical periods of growth and development," said Sanjay Keswani, M.D., Chief Medical Officer, Neurocrine Biosciences. "The initiation of this Phase 2 study reflects our commitment to evaluating crinecerfont as a potential treatment option that could reduce the need for long-term supraphysiologic glucocorticoid use and help mitigate the associated risks in this vulnerable, very young population."

CAH is typically identified at or shortly after birth and can lead to life-threatening adrenal crises due to the underlying adrenal insufficiency, as well as androgen excess and consistent dosing of supraphysiologic glucocorticoids – complications for which there are no approved therapies in children under 4 years of age. Neurocrine is conducting this pediatric study under an FDA Pediatric Written Request.

The Phase 2 open-label, single-arm study consists of a 24-week treatment period with a primary objective of assessing the safety and tolerability of crinecerfont in 20 participants aged 3 months to under 4 years with classic CAH. Secondary objectives include evaluation of the pharmacokinetics and pharmacodynamic effects of crinecerfont on hormone biomarkers. This study is expected to support a planned supplemental New Drug Application to expand the approved U.S. indication to include patients less than 4 years of age. Additional information about the trial, including eligibility criteria, can be found at ClinicalTrials.gov.

Separately, Neurocrine achieved target enrollment for a Phase 2 study in the European Union to evaluate the safety and tolerability of crinecerfont in children from birth to under 2 years of age with classic CAH. For more information, visit ClinicalTrials.gov.

Crinecerfont was approved by the U.S. Food and Drug Administration in 2024, marking the first therapeutic advancement in more than 70 years for patients with classic CAH. It is a potent and selective oral corticotropin-releasing factor type 1 receptor (CRF_1) antagonist that reduces elevated adrenocorticotropic hormone (ACTH) secretion at the source and the resulting downstream excess adrenal androgens through a non-GC mechanism.

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 4 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 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*)

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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 our future development plans with respect to crinecerfont; the efficacy and therapeutic potential of crinecerfont in children aged 3 months to under 4 years with classic congenital adrenal hyperplasia (CAH); and the value and benefits CRENESSITY brings to adults and pediatric patients 4 years of age and older with CAH. 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 that clinical development activities may not be initiated or completed on time or at all, or may be delayed for regulatory, manufacturing, or other reasons, may not be successful or replicate previous clinical trial results, may fail to demonstrate that our product candidates are safe and effective, or may not be predictive of real-world results or of results in subsequent clinical trials; risks that regulatory submissions for our product candidates may not occur or be submitted in a timely manner; our future financial and operating performance; risks associated with our dependence on third parties for development, manufacturing, and commercialization activities for our products and product candidates, and our ability to manage these third parties; risks that the FDA or other regulatory authorities may make adverse decisions regarding our products or product candidates; risks that the potential benefits of the agreements with our collaboration partners may never be realized; risks that our products, and/or our product candidates 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 associated with U.S. federal or state legislative or regulatory and/or policy efforts which may result in, among other things, an adverse impact on our revenues or potential revenue; risks associated with potential generic entrants for our products; 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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