



Neurocrine Biosciences Presents Post Hoc Data Analysis in Congenital Adrenal Hyperplasia at ESPE 2023

September 21, 2023

SAN DIEGO, Sept. 21, 2023 /PRNewswire/ -- [Neurocrine Biosciences, Inc.](#) (Nasdaq: NBIX), today announced that it will present a new post hoc analysis of Phase 2 data of the investigational drug crinecerfont in adolescent patients with classic congenital adrenal hyperplasia (CAH) due to 21-hydroxylase deficiency (21-OHD). The analysis suggests that adolescents with classic CAH who have more elevated baseline hormone levels may have the potential for a greater response to treatment with crinecerfont and may experience a reduction in androgen levels across a broad range of glucocorticoid doses. These new data will be presented at the 61st Annual European Society for Pediatric Endocrinology ([ESPE](#)) Meeting in The Hague, Netherlands from Sept. 21–23, 2023.



In previously reported Phase 2 study data, crinecerfont treatment for 14 days led to clinically meaningful reductions of 17-hydroxyprogesterone (17-OHP), adrenocorticotrophic hormone (ACTH) and androstenedione (A4) in adolescents (ages 14–16) with CAH due to 21-OHD. Post hoc analysis of these data released today assessed whether baseline hormone concentration and glucocorticoid (GC) doses correlated with response to treatment. A strong correlation was found between baseline hormone concentration and change from baseline to Day 14 for 17-OHP, ACTH and A4, with the greatest reductions observed in participants with the highest baseline hormone levels. These data suggest adolescents with higher baseline hormone concentrations may have the potential for a greater response to treatment with crinecerfont. However, there was no correlation between baseline GC dose and treatment response, which suggests that androgen reduction might occur across a broad range of GC doses in this population.

In the Phase 2 study, crinecerfont was generally well tolerated in adolescents, with no serious adverse events or discontinuations due to adverse events. There were no safety concerns with respect to routine laboratory tests, vital signs, electrocardiograms or neuropsychiatric assessments.

The results of the post hoc analysis in adolescents were consistent with results from similar post hoc analyses of the Phase 2 study of crinecerfont in adults with classic CAH, suggesting that higher baseline hormone concentrations might predict greater response, and androgen reduction might occur across a broad range of GC doses. See the following abstract for more information: [Oral Presentation # 97 FC1.4](#); Response to Crinecerfont Treatment in Adolescents with Classic Congenital Adrenal Hyperplasia Is Correlated with Elevated Baseline Hormone Concentrations but Not Glucocorticoid Dose.

"Treating CAH in adolescents is an especially difficult task because of the need to make treatment adjustments that will minimize the risks associated with both androgen excess and exposure to supraphysiologic glucocorticoid doses. Balance can be particularly difficult to achieve in this population, where glucocorticoid dose adjustment can be made more challenging by the criticality of age-related physiological changes that occur as children enter puberty," said Eiry W. Roberts, M.D., Chief Medical Officer at Neurocrine Biosciences. "These data support the potential of crinecerfont to provide improved androgen control across a broad range of CAH patients. We plan to announce top-line data from our Phase 3 CAHtalyst™ Pediatric Study in early Q4 2023."

About Classic Congenital Adrenal Hyperplasia (CAH)

Congenital adrenal hyperplasia (CAH) refers to a group of genetic conditions that result in an enzyme deficiency that alters the production of adrenal hormones, which are essential for life. Approximately 95 percent of CAH cases are caused by a mutation that leads to deficiency of the enzyme 21-hydroxylase (21-OHD). In classic CAH, severe deficiency of this enzyme leads to an inability of the adrenal glands to produce cortisol and, in approximately 75 percent of cases, aldosterone. If left untreated, classic CAH can result in salt wasting, dehydration and even death.

There are currently no non-glucocorticoid treatments approved by the U.S. Food and Drug Administration (FDA) for classic CAH. Glucocorticoids (GCs), the current standard of care, are used not only to correct the endogenous cortisol deficiency but typically used at greater than physiologic (supraphysiologic) doses to try to suppress the high levels of corticotropin-releasing factor (CRF) and adrenocorticotrophic hormone (ACTH) that result in androgen excess. However, glucocorticoid 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 GC doses may have psychological and cognitive impact such as changes in mood and memory. Androgen excess has been associated with abnormal bone growth and development in pediatric patients, female health problems such as acne, excess hair growth and menstrual irregularities, testicular rest tumors in males and fertility issues in both sexes. To learn more about CAH, click [here](#).

About Crinecerfont

Crinecerfont is an investigational, oral, selective corticotropin-releasing factor type 1 receptor (CRF₁) antagonist being developed to reduce and control excess adrenal androgens through a steroid-independent mechanism for the treatment of CAH due to 21-hydroxylase deficiency (21-OHD). Antagonism of CRF type 1 receptors in the pituitary has been shown to decrease adrenocorticotrophic hormone (ACTH) levels, which in turn decreases the production of adrenal androgens and potentially the symptoms associated with classic CAH. Our data demonstrates that lowering androgen levels enables lower, more physiologic dosing of glucocorticoids and thus could potentially reduce the complications associated with exposure to greater than normal glucocorticoid doses in patients with classic CAH.

[Learn](#) more about crinecerfont.

About CAHtalyst™ Studies

The randomized, double-blind, placebo-controlled CAHtalyst™ Phase 3 global registrational studies were designed to evaluate the safety, efficacy and tolerability of crinecerfont in children and adolescents (ages 2–17 years of age) as well as adults (18 years of age and older) with classic congenital adrenal hyperplasia (CAH). As part of the CAHtalyst clinical trial program, participants who completed these studies were able to continue to receive crinecerfont as part of an open-label extension. On September 12, 2023, Neurocrine Biosciences announced positive top-line data from the Phase 3 CAHtalyst Adult study. Neurocrine Biosciences plans to announce top-line data from its Phase 3 CAHtalyst Pediatric study in early Q4 2023.

For more information about the CAHtalyst Adult and CAHtalyst Pediatric Phase 3 studies, please visit [ClinicalTrials.gov](#) (Adult) and [ClinicalTrials.gov](#) (Pediatric).


About Neurocrine Biosciences

Neurocrine Biosciences is a leading neuroscience-focused, biopharmaceutical company with a simple purpose: to relieve suffering for people with great needs but few options. 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, Parkinson's disease, 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](#), [Twitter](#) and [Facebook](#). (*in collaboration with AbbVie)

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Forward-Looking Statement

In addition to historical facts, this press release contains forward-looking statements that involve a number of risks and uncertainties. These statements include statements regarding the potential benefits of crinecerfont to patients and future clinical development plans. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements include the risk that crinecerfont will not be found to be safe and/or effective or may not prove to be beneficial to patients; that development activities for crinecerfont may not be completed on time or at all; risks that clinical development activities may be delayed for regulatory or other reasons, may not be successful or replicate previous and/or interim clinical trial results, or may not be predictive of real-world results or of results in subsequent clinical trials; risks that regulatory submissions for crinecerfont may not occur or be submitted in a timely manner; risks that crinecerfont may not obtain regulatory approvals; or that the U.S. Food and Drug Administration or regulatory authorities outside the U.S. may make adverse decisions regarding crinecerfont; 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 June 30, 2023. Neurocrine disclaims any obligation to update the statements contained in this press release after the date hereof.

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