# Neurocrine Biosciences Announces U.S. FDA Accepts New Drug Applications and Grants Priority Review for Crinecerfont for Pediatric and Adult Patients with CAH

July 1, 2024

- PDUFA Target Action Dates in Late December 2024
- Highly Selective $CRF_1$ Antagonist is the Potential First New Treatment for CAH in 70 Years

SAN DIEGO, July 1, 2024 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced the U.S. Food and Drug Administration (FDA) has accepted its two New Drug Applications (NDA) with Priority Review designations for crinecerfont in the treatment of children, adolescents and adults with classic congenital adrenal hyperplasia (CAH). If approved, crinecerfont would be the first new treatment option for CAH in 70 years and a first-in-class therapy, with a novel approach for the treatment of this rare and serious endocrine disorder.

The submitted crinecerfont NDAs included: the primary presentation of efficacy and safety of crinecerfont for the treatment of classic CAH as (1) a capsule formulation (NDA# 218808); and (2) as an oral solution formulation (NDA# 218820). The agency set Prescription Drug User Fee (PDUFA) target action dates of December 29 and December 30, 2024, respectively. The FDA stated it is not currently planning to hold an advisory committee meeting to discuss these applications.

Priority Review designation by the FDA accelerates the review timeline by four months – and means the agency recognizes CAH is a serious condition with high unmet medical need and crinecerfont is a treatment that provides significant benefit over current therapy. Should crinecerfont receive FDA approval, it will enable Neurocrine Biosciences to activate its Rare Pediatric Disease Designation Priority Review Voucher – a designation granted in September 2020.

"Receipt of a Priority Review reflects the FDA's agreement that CAH is a serious condition and there is an urgent need for patients to have access to new treatments," said Eiry W. Roberts, M.D., Chief Medical Officer at Neurocrine Biosciences. "Crinecerfont's compelling efficacy results and excellent safety profile support our filing, and we look forward to working with the FDA as we head toward the PDUFA dates at the end of 2024."

Crinecerfont previously was granted Orphan Drug designation in March 2019 and Breakthrough Therapy designation in December 2023.

Orphan Drug designation means the company will be exempt from paying PDUFA user fees, receive tax credits for qualified clinical trials, and has the potential of seven years of market exclusivity should crinecerfont be approved.

Breakthrough Therapy designation is a process developed by the FDA to expedite development and review of drugs that are intended to treat a serious condition and where clinical evidence indicates that the potential drug may demonstrate substantial improvement over available therapy on a clinically significant endpoint(s). Breakthrough Therapy designation was granted based on the strong results and excellent safety profile seen from the CAHtalyst™ Phase 3 Pediatric and Adult study data announced in fall 2023 and published in June.

CAHtalyst Phase 3 data results in pediatric and adult patients with CAH due to 21-OHD were published in online issues of *The New England Journal of Medicine* on June 2 and June 1, respectively, and presented at ENDO 2024.

**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 hormones which are essential for life. Approximately 95% of CAH cases are caused by a mutation that leads to deficiency of the enzyme 21-hydroxylase (21-OHD). Severe deficiency of this enzyme leads to an inability of the adrenal glands to produce cortisol and, in approximately 75% of cases, aldosterone. If left untreated, CAH can result in salt wasting, dehydration, and even death.

Glucocorticoids (GCs) are currently 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, 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. Adrenal 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 and the CAHtalyst™ Studies**
Crinecerfont is an investigational, oral, selective corticotropin-releasing factor type 1 receptor ($CRF_1$) antagonist being developed to reduce and

control excess adrenocorticotropic hormone (ACTH) and adrenal androgens through a glucocorticoid-independent mechanism for the treatment of congenital adrenal hyperplasia (CAH) due to 21-hydroxylase deficiency. 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. Crinecerfont study data demonstrate that lowering adrenal androgen levels enables lower, more physiologic dosing of glucocorticoids to manage androgen excess and thus could potentially reduce the complications associated with exposure to greater than normal glucocorticoid doses in patients with CAH.

The CAHtalyst™ Pediatric and Adult Phase 3 global registrational studies are designed to evaluate the safety, efficacy, and tolerability of crinecerfont in children and adolescents, and adults respectively, with congenital adrenal hyperplasia due to 21-hydroxylase deficiency. The primary portions of the CAHtalyst Phase 3 studies have completed and enrollment is closed, while the open-label treatment portions of both studies are ongoing. Data from the CAHtalyst Pediatric and CAHtalyst Adult Phase 3 studies supported two New Drug Application submissions to the U.S. Food and Drug Administration in April 2024.

To learn more about crinecerfont and the CAHtalyst studies, click here.

**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, 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, 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 (formerly Twitter), and Facebook.
(**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 crinecerfont. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements include: the crinecerfont NDAs may not obtain regulatory approval, such approval may be delayed, or may not receive the benefits associated with priority review; additional regulatory submissions may not occur or be submitted in a timely manner; the FDA may make adverse decisions regarding crinecerfont; crinecerfont may not be found to be safe and/or effective or may not prove to be beneficial to patients; development activities for crinecerfont may not be completed on time or at all; 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; competitive products and technological changes that may limit demand for our products; uncertainties relating to patent protection and intellectual property rights of third parties; our dependence on third parties for development and manufacturing activities related to crinecerfont, and our ability to manage these third parties; our future financial and operating performance; risks and uncertainties associated with the commercialization of 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, 2024. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof.

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