# Neurocrine Biosciences to Present Phase 3 Baseline Characteristics Data from the CAHtalyst™ Program of Crinecerfont in CAH, and Data for Modified-Release Hydrocortisone in Primary Adrenal Insufficiency and CAH Studies at ECE 2024

May 7, 2024

- CAHtalyst™ Pediatric and CAHtalyst™ Adult Baseline Characteristics Data in Congenital Adrenal Hyperplasia
- Phase 2 (CHAMPAIN) Clinical Study Data for Modified-Release Hydrocortisone (Chronocort®/Efmody®) in Adrenal Insufficiency
- Phase 3 Extension Study Data for Modified-Release Hydrocortisone in Congenital Adrenal Hyperplasia

SAN DIEGO, May 7, 2024 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced that it will present key information from its neuroendocrinology pipeline, including baseline characteristics data from its CAHtalyst™ Program of crinecerfont in congenital adrenal hyperplasia (CAH), as well as data from its modified-release hydrocortisone studies in primary adrenal insufficiency and CAH, at the European Congress of Endocrinology 2024 meeting in Sweden, May 11–14.

Neurocrine Biosciences will be presenting several abstracts and posters at ECE 2024, including:

- Baseline Characteristics of Children and Adolescents with Classic Congenital Adrenal Hyperplasia Enrolled in CAHtalyst Pediatric, a Phase 3 Study of Crinecerfont, a Corticotropin-Releasing Factor Type 1 Receptor Antagonist
  - May 13; 5:30–6:30pm (Poster# P225)
- Baseline Characteristics of Adults with Classic Congenital Adrenal Hyperplasia Enrolled in CAHtalyst Adult, a Phase 3 Study of Crinecerfont, a Corticotropin-Releasing Factor Type 1 Receptor Antagonist
  - May 14; 1:40–3:10pm (Poster# P423)
- CHAMPAIN study: Initial Results from a Phase II Study of Efficacy, Safety and Tolerability of Modified-Release Hydrocortisones: Chronocort® (Efmody®) versus Plenadren, in Primary Adrenal Insufficiency,
  - May 12; 2:00–2:40pm (Rapid Communication #RC3.4)
- Biochemical Control with Dose Reduction in Chronic Glucocorticoid Therapy over 4 Years: A Phase III Extension Study of Chronocort (Efmody®) in the Treatment of Congenital Adrenal Hyperplasia (CAH)
  - May 12; 2:00–2:40pm (Rapid Communication #RC3.1)
- Incidence of Adrenal Crisis in Congenital Adrena Hyperplasia (CAH) Patients During a Prospective Monitored Long-Term study of Modified-Release Hydrocortisone (MRHC) Capsules, (Efmody)
  - May 12; 4:20–6:00pm (Poster #P215)
- Morning Cortisol Levels in Patients with Established Primary Adrenal Insufficiency
  - May 13; 5:30– 6:30pm (Poster #P13)

**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 adrenal androgens through a glucocorticoid-independent mechanism for the treatment of congenital adrenal hyperplasia due to 21-hydroxylase deficiency. Antagonism of $CRF_1$ receptors in the pituitary has been shown to decrease adrenocorticotropic hormone levels, which in turn decreases the production of adrenal androgens and potentially the symptoms associated with CAH. Our data demonstrate that lowering adrenal 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 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.

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

**About Primary Adrenal Insufficiency**
Primary adrenal insufficiency is a chronic endocrine condition that occurs when the body does not make enough of certain adrenal hormones, including cortisol and often aldosterone. Glucocorticoids such as hydrocortisone are used to replace the missing cortisol, but typical dosing regimens do not match the natural diurnal rhythm of the body's cortisol production.

**About Modified-Release Hydrocortisone**
Diurnal Ltd. developed modified-release hydrocortisone, a preparation of hydrocortisone that has been specifically designed to replicate the natural circadian rhythm of cortisol, when given in a twice-a-day "toothbrush" regimen, (administered last thing at night before sleep and first thing in the morning on waking). In 2021, modified-release hydrocortisone (EFMODY®) received marketing authorization for the treatment of congenital adrenal hyperplasia from the Medicines and Healthcare products Regulatory Agency (MHRA) in Great Britain (England, Wales, and Scotland) and from the European Commission in the European Union. Neurocrine Biosciences acquired Diurnal Group plc. in November 2022. A new drug application for the modified-release hydrocortisone formulation has not been submitted to the U.S. Food and Drug Administration.

**About MRHC Phase 2 Study in Adrenal Insufficiency (CHAMPAIN)**
The CHAMPAIN Phase 2 clinical study compared the efficacy, safety and tolerability of twice daily DNL0200 (Chronocort), a modified-release hydrocortisone, with once daily Plenadren, a combination of immediate- and delayed-release hydrocortisone (authorized for use in the European Union), over a treatment period of up to 2 months in participants ≥18 years of age and diagnosed with primary adrenal insufficiency.

**About the Phase 3 Extension Study for MRHC in CAH (DIUR-006)**
The DIUR-006 Phase 3 open-label extension study assessed the long-term efficacy, safety and tolerability of twice-daily DNL0200 (Chronocort®), a modified-release hydrocortisone in adults with CAH. The study was performed to build on the results of feeder studies DIUR-003 (Phase 2 in adults with CAH) and DIUR-005 (EU Phase 3 Registrational Open-Label Study of Chronocort compared to standard of care in adults with CAH) and evaluate the long-term safety of Chronocort, and also its long-term efficacy in improving control of serum androgen levels (using 17-OHP and A4 as biomarkers).

**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 certain of our products. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements include: risks that regulatory submissions for our products and/or product candidates may not occur be submitted in a timely manner, or accepted for filing; our products and/or product candidates 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 our products and/or product candidates; our products and/or product candidates will not be found to be safe and/or effective or may not prove to be beneficial to patients; that development activities for our products and/or product candidates may not be completed on time or at all; 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; 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 our products and our product candidates, 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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