



Neurocrine Biosciences Presented CAHtalyt™ Pediatric Study Baseline Characteristics and CAHtalog™ Registry Data at PES 2024

May 3, 2024

- **CAHtalyt™ Pediatric Study Baseline Characteristics Data Highlight Need for Novel Treatments in Children and Adolescents with Congenital Adrenal Hyperplasia (CAH)**
- **CAHtalog™ Registry Data of Glucocorticoid Treatment Patterns in Pediatric and Adult Patients Illustrates the Challenges of Long-Term CAH Management**

SAN DIEGO, May 3, 2024 /PRNewswire/ -- [Neurocrine Biosciences, Inc.](#) (Nasdaq: NBIX), today presented the CAHtalyt™ Pediatric Phase 3 clinical study baseline characteristics data for children and adolescents with congenital adrenal hyperplasia (CAH) due to 21-hydroxylase deficiency enrolled in the study, along with CAHtalog™ Registry data assessing glucocorticoid treatment patterns in pediatric and adult patients with CAH.



These data demonstrate the limitations of current CAH treatment approaches in pediatric patients, comorbidities associated with the condition and/or current treatment, including obesity, advanced bone age, and early puberty, and the difficulty in managing the disease effectively as CAH patients age into adults. These new data were presented at the [Pediatric Endocrine Society 2024 Annual Meeting](#) in Chicago.

CAHtalyt Phase 3 Pediatric Study Baseline Characteristics

Baseline characteristics of the subjects who enrolled in the CAHtalyt Pediatric Phase 3 study were presented (Poster# 56). The study enrolled 103 subjects 4 to 17 years of age with a medically confirmed diagnosis of CAH due to 21-hydroxylase deficiency, with 52% male, mean age 12 years old, and majority in Tanner stages 3-5. There was evidence indicating inadequate adrenal androgen control in many of these patients despite supraphysiologic glucocorticoid dosing. At baseline, more than a third of the participants reported comorbidities of advanced bone age, early puberty, and obesity. Hirsutism (excessive hair growth, 12%) and irregular menses (12%) were reported in females, and testicular adrenal rest tumors were identified in more than a third of males.

"At baseline, many participants in the CAHtalyt Pediatric study showed clinical evidence of elevated glucocorticoid doses and adrenal androgen excess. Many exhibited obesity, advanced bone age, and early puberty, all of which can negatively impact development in childhood and adolescence, and lead to further harm in adulthood," said Eiry W. Roberts M.D., Chief Medical Officer at Neurocrine Biosciences. "With adrenal androgen levels elevated despite supraphysiologic glucocorticoid doses, it's clear that there is a significant need for a new approach to treat this condition."

In 2023, Neurocrine Biosciences announced positive top-line data from the CAHtalyt Pediatric and CAHtalyt Adult Phase 3 clinical studies evaluating the efficacy, safety, and tolerability of crinecerfont in pediatric and adult patients with CAH due to 21-hydroxylase deficiency. The data from both studies supported two New Drug Applications that were submitted to the U.S. Food and Drug Administration in April 2024.

CAHtalog Patient Registry: Glucocorticoid Treatment Patterns

Neurocrine Biosciences also presented glucocorticoid treatment patterns from a recent cohort of pediatric and adult patients participating in the CAHtalog Registry (Poster# 51). Data from 42 pediatric and 32 adult patients with median duration of observation between 10 to 13 years of age were analyzed.

Glucocorticoid treatment patterns seen in the CAHtalog Registry data indicate patients are being treated with GC doses that are at or above the upper end of the ranges recommended in the Endocrine Society Guidelines.

"The CAHtalog Registry data showed that a vast majority of patients had higher GCs and/or a loss of androstenedione control at some point, or multiple points, in their journey living with CAH," said Oksana Lekarev, D.O., Associate Professor of Clinical Pediatrics, Co-Medical Director of the Weill Cornell Medicine Comprehensive Care Center for Congenital Adrenal Hyperplasia and an Associate Attending Pediatrician at New York-Presbyterian/Weill Cornell Medical Center and member of the CAHtalog Scientific Advisory Board. "These analyses underscore that disease control can vary widely and highlights the difficulty in achieving optimal adrenal androgen control in many patients despite supraphysiologic GC dosing."

Neurocrine Biosciences abstracts presented at the meeting included:

- **Baseline Characteristics of Children and Adolescents with Classic Congenital Adrenal Hyperplasia Enrolled in CAHtalyt Pediatric, a Phase 3 Study of Crinecerfont, a Corticotropin-Releasing Factor Type 1 Receptor Antagonist, May 3; 12:15–1:45pm (Poster# 56)**
- **Glucocorticoid Treatment Patterns in Pediatric and Adult Patients with Classic Congenital Adrenal Hyperplasia: Results**

from the CAHtalog Registry, May 3; 12:15–1:45pm (Poster# 51)

- Natural History of Classic Congenital Adrenal Hyperplasia: Results from Pediatric and Adult Patients in the CAHtalog Registry, May 3; 12:15–1:45pm (Poster# 60)

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. Severe deficiency of this enzyme can lead 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 adrenocorticotrophic 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 Crinercerfont

Crinercerfont is an investigational, oral, selective corticotropin-releasing factor type 1 receptor (CRF₁) 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 CRF₁ receptors in the pituitary has been shown to decrease adrenocorticotrophic 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 replacement glucocorticoid doses in patients with CAH.

To learn more about crinercerfont, click [here](#).

About the CAHtalyt™ Phase 3 Studies

The CAHtalyt™ Pediatric and Adult Phase 3 global studies are the largest registrational studies conducted to date to evaluate the safety, efficacy, and tolerability of crinercerfont in children and adolescents, and adults respectively, with congenital adrenal hyperplasia due to 21-hydroxylase deficiency. The double-blind portions of the CAHtalyt Phase 3 studies have completed enrollment and are closed, while open-label treatment in both studies is ongoing.

For more information about the CAHtalyt Pediatric Phase 3 study, please visit [ClinicalTrialsPediatric.gov](#).

For more information about the CAHtalyt Phase 3 study in adults (ages 18 years of age and older), please visit [ClinicalTrialsAdult.gov](#).

About the CAHtalog™ Registry

In 2021, the CARES Foundation, Neurocrine Biosciences and PicnicHealth partnered to establish the CAHtalog (Congenital Adrenal Hyperplasia: Patient and Clinical Outcomes in Real-World Practice Settings) Registry. The CAHtalog Registry is a U.S. based CAH patient registry, or collection of clinical patient data, for patients with CAH due to 21-hydroxylase deficiency. The database was developed to support patient-centered clinical research to enhance the scientific community's foundational knowledge about CAH, and ultimately help patients who live with the condition. For more information about the CAHtalog Registry, please visit [CAHtalog.com](#).

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)

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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 the potential benefits to be derived from crinercerfont, as well as the therapeutic potential and clinical benefits or safety profile of crinercerfont. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements include: the crinercerfont NDAs may not be accepted for filing by the FDA or may not obtain regulatory approval or such approval may be delayed; additional regulatory submissions may not occur or be submitted in a timely manner; the FDA may make adverse decisions regarding crinercerfont; crinercerfont may not be found to be safe and/or effective or may not prove to be beneficial to patients; development activities for crinercerfont 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 crinercerfont, 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.

 View original content to download multimedia: <https://www.prnewswire.com/news-releases/neurocrine-biosciences-presented-cahtalyst-pediatric-study-baseline-characteristics-and-cahtalog-registry-data-at-pes-2024-302135660.html>

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