Neurocrine Biosciences to Present Data on Treatment Patterns and Unmet Needs in Adult and Pediatric Patients Living with Classic Congenital Adrenal Hyperplasia at AMCP 2022

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SAN DIEGO, March 28, 2022 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced that it will present new data on treatment patterns and unmet needs in adult and pediatric patients with classic congenital adrenal hyperplasia (CAH) at the Academy of Managed Care Pharmacy (AMCP) 2022 annual meeting in Chicago on March 29–April 1. The three studies being presented evaluate the treatment patterns, healthcare-related costs, medication preferences, and unmet needs of patients with classic CAH.

"There are currently no non-steroidal U.S. FDA-approved treatments for classic CAH, which means management of the condition can be challenging for patients and caregivers alike," said Eiry W. Roberts, M.D., Chief Medical Officer at Neurocrine Biosciences. "The data we are presenting at AMCP 2022 reinforce the challenges of managing classic CAH among patients living with this condition, with a treatment paradigm that has remained unchanged for more than 60 years. These studies underscore our commitment to improving the scientific and medical community's understanding of classic CAH and the limitations of the current standard of care, and are instrumental in guiding our efforts to advance new treatment options."

Classic CAH due to 21-hydroxylase deficiency (21-OHD) is a rare autosomal recessive condition characterized by cortisol deficiency and elevated adrenocorticotropic hormone (ACTH) secretion, resulting in excess androgen production. The current standard of care is glucocorticoid (GC) therapy to replace endogenous cortisol deficiency; however, supraphysiologic GC doses are often needed to reduce elevated ACTH secretion and excess androgen production. Monitoring and titrating GC treatment remains a major clinical challenge due to the competing priorities of avoiding the clinical consequences of excess androgen from GC undertreatment, including accelerated growth before puberty that results in height below genetic potential, virilization and menstrual irregularities in females, testicular adrenal rest tumors in males, and fertility problems in both sexes in adulthood, while minimizing the risk of the well-recognized sequelae and complications of chronic GC overtreatment, including metabolic abnormalities, bone loss, growth impairment, and iatrogenic Cushing's syndrome.

Neurocrine Biosciences is currently developing crinecerfont, an investigational, oral, non-steroidal, selective corticotropic-releasing factor type 1 (CRF_1) receptor antagonist for the treatment of classic CAH due to 21-OHD. Neurocrine Biosciences is currently conducting two Phase 3 global registrational studies of crinecerfont in adults (ages 18 years and older) and children and adolescents (ages 2 to 17 years old) with classic CAH, called the CAHtalyst ™ Adult and CAHtalyst ™ Pediatric studies, respectively.

Presentations at AMCP 2022 include:

- "Treatment Patterns and Unmet Needs in Adults with Classic Congenital Adrenal Hyperplasia: A Modified Delphi Consensus Study" (Poster #E26)
- "Healthcare Resource Utilization in Patients with Classic Congenital Adrenal Hyperplasia" (Poster #E24)
- "Patient Medication Preferences in Classic Congenital Adrenal Hyperplasia: A Discrete Choice Experiment" (Poster #E25)

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. Approximately 95% of CAH cases are caused by a mutation that leads to deficiency of the enzyme 21-hydroxylase. In classic CAH, 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, classic CAH can result in salt wasting, dehydration, and even death. Even with glucocorticoid treatment, high levels of adrenocorticotropic hormone (ACTH) from the pituitary gland result in excess androgen production leading to virilization and menstrual irregularities in females. Both males and females with classic CAH can experience problems with growth and development in childhood including early puberty, short stature or height below genetic potential, and fertility problems in adulthood.

There are currently no non-steroidal treatments approved by the U.S. Food and Drug Administration (FDA) for classic CAH. Glucocorticoids, the current standard of care, are used to correct the endogenous cortisol deficiency and to try to reduce the high ACTH levels and androgen excess. However, the dose of steroid use required to try to control androgen excess is generally well above the normal physiological level of cortisol, and the chronic duration of high steroid dose administration can result in serious and common complications of steroid excess, including metabolic abnormalities, bone loss, growth impairment, and iatrogenic Cushing's syndrome.

To learn more about CAH, click here.

About Crinecerfont
Crinecerfont is an investigational, oral, nonsteroidal, selective corticotropin-releasing factor type 1 (CRF_1) receptor antagonist under evaluation for the treatment of classic CAH due to 21-hydroxylase deficiency (21-OHD). Antagonism of CRF_1 receptors in the pituitary has been shown to decrease ACTH levels, which in turn decreases the production of adrenal steroids, including androgens, and potentially the symptoms associated with classic CAH. Research also suggests that lowering androgen levels may enable lower dosing of glucocorticoids and thus potentially reduce the long-term exposure to greater than normal glucocorticoid doses in patients with classic CAH.

To learn more about crinecerfont, click here.

About CAHtalyst ™ Studies
Neurocrine Biosciences is currently conducting two Phase 3 global registrational studies of crinecerfont in adults (ages 18 years and older) and children and adolescents (ages 2 to 17 years old) with classic CAH.

For more information about the adult CAHtalyst ™ Phase 3 study, please visit cahtalyst.cahstudies.com and ClinicalTrials.gov.

For more information about the pediatric CAHtalyst ™ Phase 3 study, please visit cahtalystpeds.cahstudies.com and ClinicalTrials.gov.

About Neurocrine Biosciences
Neurocrine Biosciences is a 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, Parkinson's disease, endometriosis*, and uterine fibroids*, as well as over a dozen mid- to late-stage clinical programs in multiple 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. (**in collaboration with AbbVie*)

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, but are not limited to, 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: our future financial and operating performance; risks and uncertainties associated with the scale and duration of the COVID-19 pandemic and resulting global, national, and local disruptions, 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-K for the year ended December 31, 2021. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof.

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