Neurocrine Biosciences Honored at CARES Foundation 22nd Anniversary Gala

June 6, 2022

SAN DIEGO, June 6, 2022 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) was honored by the CARES Foundation for its ongoing research and development of novel therapies for patients with classic congenital adrenal hyperplasia (CAH). The CARES Foundation, Inc. is the only U.S.-based nonprofit organization solely dedicated to improving the lives of the CAH patient community and seeks to advance quality health care. Neurocrine Biosciences' Chief Research Officer, Dimitri Grigoriadis, Ph.D., accepted the Corporate Partner Award at the CARES 22nd Anniversary Gala held at Sony Pictures Studios in Culver City, CA on June 4.

"This award recognizes the members of our outstanding research and development, and clinical development teams, who are leading our effort to advance a potential new treatment option for classic CAH," said Kevin Gorman, Ph.D., Chief Executive Officer. "Thanks to the vision of our co-founder Dr. Wylie Vale and the leadership of our Chief Research Officer Dr. Dimitri Grigoriadis, our investigational compound crinecerfont is currently in Phase 3 registrational studies in adults, adolescents, and children. Crinecerfont has the potential to help the thousands of people living with classic CAH, and we're looking forward to reporting top-line data from these studies in 2023."

Classic CAH is a genetic disorder that impacts the lives of up to 30,000 people in the United States and 50,000 people in Europe. Wylie Vale, Ph.D., isolated corticotropin-releasing factor (CRF), which plays an integral part in the hypothalamic-pituitary-adrenal (HPA) axis that is disrupted in classic CAH. He began his career making major contributions to the discovery of hypothalamic peptides in the lab of Roger Guillemin, Ph.D., who ultimately received the 1977 Nobel Prize in Physiology and Medicine. Dimitri Grigoriadis, Ph.D., has spent most of his career studying CRF, including years working with Dr. Vale. His research was the basis for the development of crinecerfont, an oral, non-steroidal, selective corticotropin-releasing factor type 1 (CRF_1) receptor antagonist for the treatment of classic CAH due to 21-hydroxylase deficiency (21-OHD).

For more than 60 years, glucocorticoid treatment (typically at supraphysiologic doses) has been the standard treatment to reduce elevated adrenal androgens in patients with classic CAH. There are currently no non-steroidal U.S. Food and Drug Administration (FDA)-approved treatments for classic CAH.

Neurocrine Biosciences is currently conducting two Phase 3 global registrational studies of crinecerfont in adults (18 years of age and older) and children and adolescents (2 to 17 years of age) with classic CAH, called the CAHtalyst ™ Adult and CAHtalyst ™ Pediatric studies, respectively. As part of the CAHtalyst clinical trial program, participants who complete these trials will be able to continue to receive crinecerfont as part of an open-label extension.

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 results 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 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, increased cardiovascular risk, bone loss, fractures, growth impairment, and increased infection risk.

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 could decrease the production of adrenal androgens and potentially the symptoms associated with classic CAH. Research also suggests that lowering androgen levels may enable lower, more physiologic dosing of glucocorticoids and thus potentially reduce the complications associated with 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 (18 years of age and older) and children and adolescents (ages 2 to 17 years of age) 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.

As part of the CAHtalyst ™ clinical trial program, participants who complete these trials will be able to continue to receive crinecerfont as part of an open-label extension.

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, Twitter, and Facebook. (**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-Q for the quarter ended March 31, 2022. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof.

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