Neurocrine Biosciences Launches WHAT THE C@H?! Educational Initiative to Support Congenital Adrenal Hyperplasia Community

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- *WHAT THE C@H?! provides a platform for the congenital adrenal hyperplasia (CAH) community to find educational information, share experiences and learn about current research.*

SAN DIEGO, April 24, 2024 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced the launch of WHAT THE C@H?!, an educational initiative that aims to close the gap in the need for helpful information about congenital adrenal hyperplasia (CAH) and acknowledges the frustrations and challenges experienced by the community in managing the condition.

"We are proud to introduce this effort to recognize the significant physical and emotional burden individuals and their families experience living with CAH," said Eiry W. Roberts, M.D., Chief Medical Officer, Neurocrine Biosciences. "We hope this educational initiative, which was informed by insights from the community, is helpful to those navigating and managing this difficult condition."

WHAT THE C@H?! was created for adults and adolescents living with CAH, parents of children with CAH and providers who treat the condition. Through a form on the website, people can submit their WHAT THE C@H?! moment, a challenging experience of living with or managing the condition. The website features educational information and offers patients and caregivers a space to share their CAH stories with others.

CAH is a rare, genetic condition that results in an enzyme deficiency that alters the production of adrenal hormones which are essential for life.[1] CAH is typically identified at or soon after birth and is associated with adrenal insufficiency that can lead to life-threatening adrenal crises and androgen excess.[1] People with CAH seek to achieve balance between cortisol deficiency and androgen excess through treatment with glucocorticoids, which have remained the standard for the past 70 years.[1] However, side effects of treatment can be difficult to detect or distinguish from the symptoms of CAH itself, making it challenging to determine an appropriate treatment plan throughout various stages of life.

"For over 70 years, treating the symptoms of CAH has presented challenges not only for people living with the condition and their families, but for healthcare providers who are often balancing trade-offs of treatment with high-dose steroids to improve care for their patients," said Dimitri E. Grigoriadis, Ph.D., Distinguished Scholar, Neurocrine Biosciences. "Neurocrine has a deep history in advancing research in CAH and is proud to launch this educational initiative as part of our ongoing commitment to supporting the community."

"WHAT THE C@H?! provides an empowering and supportive website for individuals living with CAH and parents of children with the condition where they can express themselves, share their stories and learn about other people's experiences," said Dina Matos, Executive Director at CARES Foundation. "It's a unique educational platform that we feel will encourage the community to be their own best advocates by having active and ongoing conversations with their healthcare providers about their symptoms and treatment plan."

The WHAT THE C@H?! healthcare provider-facing website includes interactive educational resources on current CAH monitoring and management practices, in addition to an overview of the potential of corticotropin-releasing factor (CRF_1) receptors in CAH.

Dozens of interviews were conducted with people living with CAH, their family members and healthcare providers, in addition to extensive market research, including 63 patients and 124 healthcare providers, to inform the educational initiative.

For more information and to share your WHAT THE C@H?! moment, visit WhatTheCAH.com and follow the initiative on Facebook: What The CAH?!.

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 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), the current standard of care, are 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 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. 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 Corticotropin-Releasing Factor (CRF)

Corticotropin-releasing factor (CRF) is a neuropeptide that regulates the secretion of adrenocorticotropic hormone (ACTH) by the pituitary gland. The neuropeptide was discovered, isolated and identified by the late Wylie W. Vale, PhD., a founder of Neurocrine Biosciences who led the Clayton Foundation Laboratories for Peptide Biology and was the Helen McLoraine Chair in Molecular Neurobiology at the Salk Institute. Dr. Vale mapped the key role CRF plays in the regulation of the stress response. In the 95% of CAH cases caused by a mutation in the 21-hydroxylase enzyme, severe enzyme deficiency results in little or no production of the stress hormone cortisol, which can lead to adrenal crisis. The lack of cortisol production also causes a break in the feedback loop that would normally regulate the body's secretion of CRF, and as a result ACTH, which maintains adrenal androgen production within normal ranges.

About CARES Foundation

CARES Foundation is the only organization in the United States solely dedicated to the congenital adrenal hyperplasia (CAH) community. Founded in 2000, CARES represents approximately 8,000 affected individuals, families and healthcare professionals in the U.S. and more than 70 countries. To learn more, visit www.caresfoundation.org.

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 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 Company's plans to advance a platform that may help address the needs of people affected by CAH, and the value that such a platform may bring to the community. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are: whether the Company can successfully advance a platform that may help address the needs of people affected by CAH; risks and uncertainties associated with the development of the Company's products; risks that the Company's products may be precluded from development by the proprietary rights of third parties, or have unintended side effects or adverse reactions; risks and uncertainties relating to competitive products and technological changes that may limit demand for the Company's products; risks associated with our dependence on third parties for development and manufacturing activities related to the Company's products, and our ability to manage these third parties; risks that the FDA or other regulatory authorities may make adverse decisions regarding our products; risks associated with potential generic entrants for 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 annual report on Form 10-K for the year ended December 31, 2023. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof.

REFERENCE

1. Congenital adrenal hyperplasia. National Organization for Rare Disorders. Updated June 8, 2023. Accessed February. 8, 2024 https://rarediseases.org/rare-diseases/congenital-adrenal-hyperplasia/

View original content to download multimedia:https://www.prnewswire.com/news-releases/neurocrine-biosciences-launches-what-the-ch-educational-initiative-to-support-congenital-adrenal-hyperplasia-community-302125250.html

SOURCE Neurocrine Biosciences, Inc.

Neurocrine Biosciences, Inc., Media, Aimee White, 1-858-354-7865, media@neurocrine.com; Investors, Todd Tushla, 1-858-617-7143, ir@neurocrine.com