Neurocrine Biosciences Announces U.S. FDA Accepts Supplemental New Drug Application for Valbenazine as a Treatment for Chorea Associated with Huntington Disease

December 22, 2022

- **Prescription Drug User Fee Act (PDUFA) Target Action Date Set for August 20, 2023**

SAN DIEGO, Dec. 22, 2022 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX), a leading neuroscience-focused biopharmaceutical company, today announced that the U.S. Food and Drug Administration (FDA) has accepted its supplemental New Drug Application (sNDA) for valbenazine as a treatment for chorea associated with Huntington disease (HD). The agency set a Prescription Drug User Fee Act (PDUFA) target action date of August 20, 2023.

The sNDA filing included data from the KINECT™-HD Phase 3 study and the on-going KINECT™-HD2 open-label study of valbenazine in adults with chorea associated with Huntington disease. Huntington disease is a hereditary, progressive neurodegenerative disorder. Approximately 90% of adults with HD experience chorea, an abnormal involuntary movement disorder.

"This sNDA filing advances our effort to bring a potential new treatment option to the many thousands of people experiencing chorea associated with Huntington disease in the U.S.," said Eiry W. Roberts, M.D. Chief Medical Officer at Neurocrine Biosciences. "We look forward to working with the FDA as it reviews our filing."

The sNDA for valbenazine for the treatment of chorea associated with Huntington disease is supported by data from two clinical studies, including the Phase 3 KINECT-HD study and the on-going KINECT-HD2 open-label rollover study, with more than 150 patients with Huntington disease. The KINECT-HD study was a Phase 3, randomized, double-blind placebo-controlled study to evaluate the efficacy, safety, and tolerability of valbenazine as a treatment for chorea associated with Huntington disease. Approximately 128 adults 18 to 75 years of age who had been diagnosed with manifest HD and who had chorea symptoms were enrolled in the study and received once daily valbenazine or placebo for 12 weeks. KINECT-HD2 is an open-label study of approximately 150 patients for continuing valbenazine administration for the treatment of chorea associated with Huntington disease for up to 156 weeks. The primary endpoint in both studies was the change from baseline to maintenance in the Unified Huntington's Disease Rating Scale (UHDRS) Total Maximal Chorea (TMC) Score. Data from these studies along with the results from a PK study were included in the sNDA submission.

Enrollment continues in the KINECT-HD2 open-label study to evaluate the long-term safety and tolerability of valbenazine for the treatment of chorea in Huntington disease.

About the KINECT-HD Study

KINECT-HD is a Phase 3, randomized, double-blind, placebo-controlled study designed to: evaluate the efficacy of valbenazine as a once-daily treatment to reduce chorea associated with Huntington disease (HD) and evaluate the safety and tolerability of valbenazine in patients with HD. The study enrolled 128 adults 18 to 75 years of age who have been diagnosed with manifest HD and who have sufficient chorea symptoms to meet study protocol criteria. For more information on this KINECT-HD study, please visit www.huntingtonstudygroup.org.

About KINECT-HD2

KINECT-HD2 is an open-label study to evaluate the long-term safety and tolerability of valbenazine in patients with chorea associated with Huntington disease (HD). The 156-week study will enroll up to 150 adults 18 to 75 years of age who have been diagnosed with motor manifest HD and who have sufficient chorea symptoms to meet study protocol criteria. For more information on the KINECT-HD2 study, please visit www.huntingtonstudygroup.org or clinicaltrials.gov.

About Chorea associated with Huntington Disease

Huntington disease (HD) is a hereditary progressive, ultimately fatal neurodegenerative disorder in which neurons within the brain break down, resulting in motor, cognitive and psychiatric symptoms. Symptoms generally appear between the ages of 30 to 50 and worsen over a 10- to 25-year period. Many people with HD experience chorea, an abnormal involuntary movement disorder, characterized by irregular and unpredictable movements. Chorea can affect various body parts and interfere with motor coordination, gait, posture, swallowing, and speech. HD is estimated to affect approximately 40,000 adults in the U.S., with more than 200,000 at risk of inheriting the disease.

About Huntington Study Group

Founded in 1993, the Huntington Study Group (HSG), a global not-for-profit organization, together with its wholly owned for-profit subsidiary, HSG Clinical Research, Inc., designs, implements, manages, and conducts clinical research trials. The HSG, a leader in conducting clinical trials for HD, has more than 800 HD experts at over 130 HSG Credentialed Research Sites worldwide. The mission of the HSG is seeking treatments that make a difference for those affected by HD. The HSG also offers educational services like CME4HD™ for healthcare professionals and care providers on treating patients with HD. For more information, visit our website **www.huntingtonstudygroup.org.**

The KINECT-HD study was conducted in cooperation with the HSG and the Clinical Trials Coordination Center (CTCC) at the University of Rochester Medical Center's Center for Health + Technology (CHeT). For more information, visit the CTCC website **https://www.urmc.rochester.edu/health-technology/our-expertise/clinical-trials-coordination.aspx.**

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 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 the Company's products and product candidates. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements include: risks that valbenazine may not obtain regulatory approval for chorea in Huntington disease (HD), or that the U.S. Food and Drug Administration or regulatory authorities outside the U.S. may make adverse decisions regarding valebenazine; risks and uncertainties associated with valbenazine development for chorea in Huntington disease (HD); risks that valbenazine development activities may not be completed or may be delayed for regulatory or other reasons, may not be successful or replicate previous clinical trial results, may fail to demonstrate that valbenazine is safe, tolerable or effective in the chorea in Huntington disease (HD) population, or may not be predictive of real-world results or of results in subsequent clinical trials; risks that regulatory submissions may not occur or be submitted in a timely manner; risks that valbenazine may have unintended side effects, adverse reactions or incidents of misuse; risks associated with the Company's dependence on third parties for development and manufacturing activities related to valbenazine; risks and uncertainties relating to competitive products and technological changes that may limit demand for valbenazine; risks and uncertainties associated with the scale and duration of the COVID-19 pandemic and resulting global, national, and local disruptions; 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 September 30, 2022. 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-announces-us-fda-accepts-supplemental-new-drug-application-for-valbenazine-as-a-treatment-for-chorea-associated-with-huntington-disease-301709335.html

SOURCE Neurocrine Biosciences, Inc.

Neurocrine Biosciences, Inc. Media Linda Seaton, 1-858-617-7292, media@neurocrine.com; Investors: Todd Tushla, 1-858-617-7143, ir@neurocrine.com