Neurocrine Biosciences Announces Positive Phase 3 Data for KINECT-HD Study Evaluating Valbenazine for Chorea Associated with Huntington Disease

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- Highly Statistically Significant Reduction in Chorea Movements (p < 0.0001) as Measured by the Unified Huntington's Disease Rating Scale (UHDRS®) Total Maximal Chorea (TMC) Score

- Placebo-Adjusted Mean Reduction in TMC Score of 3.2 Units in Valbenazine-Treated Patients

- Company Plans to Submit Supplemental New Drug Application to U.S. Food and Drug Administration in 2022

SAN DIEGO, Dec. 7, 2021 /PRNewswire/ -- Neurocrine Biosciences (Nasdaq: NBIX) today announced positive top-line data from its Phase 3 KINECT-HD study evaluating the efficacy, safety and tolerability of valbenazine, a selective vesicular monoamine transporter 2 (VMAT2) inhibitor being investigated as a once-daily treatment in adults with chorea associated with Huntington disease (HD). The study met the primary endpoint of reduction in severity of chorea, the cardinal motor feature in Huntington disease, as measured by change in the Unified Huntington's Disease Rating Scale (UHDRS®) Total Maximal Chorea (TMC) score from baseline to the average score at weeks 10 and 12.

In the randomized, double-blind, placebo-controlled KINECT-HD study, treatment with valbenazine resulted in a placebo-adjusted mean reduction in the TMC score of 3.2 units (p < 0.0001), indicating a highly statistically significant improvement in chorea. The TMC score is part of the motor assessment of the UHDRS® and measures chorea in seven different body parts, including the face, oral-buccal-lingual region, trunk and each limb independently. The TMC score is the sum of the individual scores and ranges from 0 to 28. The secondary endpoints of Clinical Global Impression of Change (CGI-C) Response Status and Patient Global Impression of Change (PGI-C) Response Status were also statistically significant in favor of valbenazine treatment.

The treatment emergent adverse events observed in this trial were consistent with the known safety profile of valbenazine. No suicidal behavior or worsening of suicidal ideation was observed in the valbenazine-treated subjects in this study. Data from the Phase 3 KINECT-HD study will be presented at a medical conference in 2022.

"The positive results of the KINECT-HD study move us closer to bringing valbenazine as a potential treatment option to patients in the U.S. living with chorea, one of the most common symptoms of Huntington disease," said Eiry W. Roberts, M.D., Chief Medical Officer at Neurocrine Biosciences. "We are immensely grateful to our partners at the Huntington Study Group and the Clinical Trials Coordination Center at the University of Rochester, New York, who were instrumental in completing this study, as well as the study participants and the families and caregivers who supported them. We will review the complete data and begin preparing a supplemental new drug application (sNDA) for submission to the U.S. Food and Drug Administration next year. In the meantime, we will continue dosing in the KINECT-HD2 study, which is evaluating the long-term safety and tolerability of valbenazine in this same patient population."

HD impacts an estimated 30,000 adults in the United States. Chorea, an involuntary movement disorder characterized by irregular and unpredictable movements, is one of the most common symptoms, affecting roughly 90% of those diagnosed with Huntington disease over the course of disease progression.

"We are energized by these positive data and grateful to have been part of a study that has advanced a potential new therapy for people living with chorea associated with Huntington disease," said Andrew Feigin, M.D., Chair of the Huntington Study Group and Professor, Department of Neurology at NYU Grossman School of Medicine. "We look forward to continuing our work with Neurocrine Biosciences through the KINECT-HD2 study and working toward our goal of benefiting the lives of those living with this condition."

"We are incredibly grateful to the KINECT-HD participants for contributing the success of this important clinical trial," said Erin Furr-Stimming, M.D., FAAN, Principal Investigator, Huntington Study Group and Professor of Neurology at McGovern Medical, UTHealth Houston.

KINECT-HD2 is an 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 motor 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 112-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, a troublesome 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 30,000 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 dedicated to discovering, developing and delivering life-changing treatments for people with serious, challenging and under-addressed neurological, endocrine and psychiatric disorders. The company's diverse portfolio includes FDA-approved treatments for tardive dyskinesia, Parkinson's disease, endometriosis*, uterine fibroids* and clinical programs in multiple therapeutic areas. For nearly three decades, Neurocrine Biosciences has specialized in targeting and interrupting disease-causing mechanisms involving the interconnected pathways of the nervous and endocrine systems. For more information, visit neurocrine.com, and follow the company on LinkedIn. (*in collaboration with AbbVie)

Neurocrine Biosciences Forward-Looking Statements
In addition to historical facts, this press release contains forward-looking statements that involve a number of risks and uncertainties. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are: risks and uncertainties associated with valbenazine development for chorea in Huntington disease (HD), that valbenazine development activities may not be completed on time or at all; 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 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 valbenazine; risks that valbenazine may be precluded from commercialization by the proprietary rights of third parties, or 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 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, 2021. Neurocrine Biosciences disclaims any obligation to update the statements contained in this press release after the date hereof.

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