Neurocrine Biosciences Presents Phase 3 Data for KINECT-HD Study Evaluating Valbenazine for Chorea Associated with Huntington Disease at AAN 2022

April 1, 2022

- **Valbenazine Met the Primary Endpoint of Significant (p<0.0001) Improvement in Chorea Severity versus Placebo as Measured by the Unified Huntington's Disease Rating Scale (UHDRS®) Total Maximal Chorea (TMC) Score, with Improvements Beginning in Week 2**

- **Clinically Meaningful Results, Demonstrated by Greater Response Rates, Seen by Clinicians (CGI-C) and Patients (PGI-C) for Valbenazine versus Placebo**

- **Valbenazine Safety Profile was Consistent with the Known Safety Profile**

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

SAN DIEGO, April 1, 2022 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced results from the Phase 3 KINECT-HD study, which demonstrated once-daily administration of valbenazine was associated with significant improvement in chorea associated with Huntington disease (HD) compared with placebo. These data (abstract #1199) will be shared as an oral presentation Tuesday, April 5th during the Emerging Science Session at the American Academy of Neurology (AAN) 2022 "Great Neuro Reunion" annual meeting in Seattle, Washington.

"Presentation of these positive data of valbenazine for chorea in Huntington disease represent a major step forward in our commitment to offering the community a potential new treatment option," said Eiry W. Roberts, M.D., Chief Medical Officer at Neurocrine Biosciences. "We are proud to work with the Huntington Study Group and the Clinical Trials Coordination Center at the University of Rochester on this program. Data from the KINECT-HD and the ongoing KINECT-HD2 study will form the basis of our supplemental new drug application (sNDA) for submission to the U.S. Food and Drug Administration later this year."

The KINECT-HD study met its primary endpoint of change in chorea severity using the TMC score of the UHDRS® from screening period baseline to maintenance period. Improvement in the TMC score was significantly greater with valbenazine versus placebo, with a placebo-adjusted mean reduction of 3.2 units vs placebo (p<0.0001). Improvements in the TMC score with valbenazine were seen as early as Week 2, and TMC scores continued to improve versus placebo throughout the dose adjustment and maintenance periods.

The secondary endpoints of Clinical Global Impression of Change (CGI-C) Response Status and Patient Global Impression of Change (PGI-C) Response Status also significantly favored valbenazine treatment. Patients achieving "much improved" or "very much improved" status were classified as responders. Using this classification, at Week 12, the response rate in the valbenazine group was 42.9% for CGI-C compared to 13.2% in the placebo group (p<0.001). The response rate at Week 12 for PGI-C was 52.7% in the valbenazine group and 26.4% in the placebo group (p<0.01). Response rates favoring valbenazine were seen as early as week 4 for CGI-C and Week 2 for PGI-C. The secondary endpoints of Neuro-QoL upper and lower extremity physical function endpoints at Week 12 were not significantly different between the treatment groups.

	Efficacy	Placebo	Valbenazine	Difference	P-values
Primary endpoint	TMC CFSPB at Maintenance*, LS Mean	-1.44	-4.60	-3.16	<0.0001
Secondary endpoints	CGI-C at Week 12, % Response Status	13.2%	42.9%	29.7%	<0.001
	PGI-C at Week 12, % Response Status	26.4%	52.7%	26.3%	<0.01
	Neuro-QoL Upper Extremity Function T Score CFB at Week 12, LS Mean	-3.00	-1.58	1.42	n.s.
	Neuro-QoL Lower Extremity Function T Score CFB at Week 12, LS Mean	0.61	-0.27	-0.88	n.s.

*Maintenance = The average of the Week 10 and Week 12 assessments
Abbreviations: LS Mean, Least squares mean; CFSPB, Change from screening period baseline (the average of screening and Day -1 assessments); n.s., non-significant

Treatment emergent adverse events, including somnolence, fatigue, fall, and akathisia, were mild to moderate and 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.

"We are pleased with this data readout and are grateful to the patients and collaborators who have helped to advance this potential treatment," said Andrew Feigin, M.D., Chief Medical Officer of the Huntington Study Group (HSG) Clinical Research, Inc., former chair of HSG, and Professor, Department of Neurology at NYU Grossman School of Medicine. "We remain committed to this patient community as we look to further our scientific understanding of the efficacy and safety of valbenazine for chorea associated with Huntington disease in the long-term trial."

The full data results from the Phase 3 KINECT-HD study will be submitted to a peer-reviewed journal later this year. The safety and tolerability of valbenazine in chorea associated with Huntington disease continues to be evaluated in KINECT-HD2, an open-label long-term safety and tolerability study. Neurocrine Biosciences and the Huntington Study Group thank the patients and investigators involved in the KINECT-HD and KINECT-HD2 clinical trials.

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 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**. (**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 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 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; the impact of the COVID-19 pandemic and efforts to mitigate its spread on the Company's business; 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-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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