Neurocrine Biosciences Receives Orphan Drug Designation for Valbenazine as a Treatment for Chorea Associated with Huntington Disease

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SAN DIEGO, May 12, 2022 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced that it has received Orphan Drug Designation from the U.S. Food and Drug Administration (FDA) for valbenazine as a treatment for Huntington disease (HD). The treatment of chorea associated with HD is within the scope of this Orphan Drug Designation. In December 2021, Neurocrine Biosciences reported 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 HD.

"Receiving an FDA Orphan Drug Designation validates our continued commitment to developing new treatment options that could benefit the lives of patients living with rare diseases, including those impacted by HD," said Kevin Gorman, Ph.D., Chief Executive Officer. "We are in the process of completing data analysis from the KINECT-HD and the ongoing KINECT-HD2 studies, which will form the basis of our supplemental new drug application (sNDA) for submission to the FDA later this year."

Enrollment is ongoing 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 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 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 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-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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