Neurocrine Biosciences Provides Update on Phase 2 Study of NBI-827104 in Pediatric Patients with Epileptic Encephalopathy with Continuous Spike-and-Wave During Sleep

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SAN DIEGO, Dec. 6, 2022 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX), a leading neuroscience-focused biopharmaceutical company, today announced that investigational NBI-827104 did not meet its primary endpoint in the Phase 2 STEAMBOAT™ study evaluating the efficacy, safety, tolerability and pharmacokinetics of NBI-827104 compared to placebo in pediatric patients with epileptic encephalopathy with continuous spike-and-wave during sleep (EE-CSWS). NBI-827104 was generally well tolerated.

EE-CSWS is a rare pediatric developmental and/or epileptic encephalopathy that is characterized by a continuous or nearly continuous spike and wave electroencephalogram (EEG) pattern during the non-rapid eye movement (NREM) phase of sleep, and regression or stagnation in cognitive, language, behavioral, or motor functions.

"While we did not meet the primary endpoint for this Phase 2 study, we remain committed to advancing care for patients living with epilepsy, including rare pediatric forms," said Eiry W. Roberts, M.D., Chief Medical Officer. "We will continue to analyze the rich data set generated from this study to determine next steps. We are grateful to everyone involved in the study, especially our study participants, their families, and our investigators."

About the STEAMBOAT Study
The STEAMBOAT Phase 2 study is a randomized, double-blind, placebo-controlled Phase 2 study that evaluated the efficacy, safety, tolerability, and pharmacokinetics of NBI-827104 when administered once daily up to 13 weeks in pediatric patients with EE-CSWS.

The primary endpoint was a reduction from baseline as compared to placebo in the ratio of spike-wave index (SWI) when measured after 6 weeks of study treatment. The SWI, a measure of the percentage of sleep affected by epileptic activity, was measured during the first hour of non-rapid eye movement (NREM) sleep by independent and centralized readings of overnight video-electroencephalograms (EEGs).

About NBI-827104
NBI-827104 is an investigational, potent, selective, and orally active brain-penetrating T-type calcium channel blocker (Ca_V 3.1, Ca_V 3.2, Ca_V 3.3) in development for the potential treatment of EE-CSWS.

Neurocrine acquired the global rights to NBI-827104 from Idorsia in May 2020.

About Neurocrine Biosciences
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, 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 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 clinical results from, and our future development plans with respect to NBI-827104. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements include: our future financial and operating performance; the risk that our product candidates will not be found to be safe and/or effective or may not prove to be beneficial to patients; that development activities for our product candidates may not be completed on time or at all; risks that clinical development activities may be delayed for regulatory or other reasons, may not be successful or replicate previous and/or interim clinical trial results, or may not be predictive of real-world results or of results in subsequent clinical trials; risks that regulatory submissions for our product candidates may not occur or be submitted in a timely manner; risks that our product candidates may not obtain regulatory approvals; or that the U.S. Food and Drug Administration or regulatory authorities outside the U.S. may make adverse decisions regarding our product candidates; 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.

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