Neurocrine Announces Positive Results from Phase III Kinect 3 Study of NBI-98854 in Tardive Dyskinesia

October 8, 2015

Study Meets Primary Endpoint, Submission of New Drug Application Planned for 2016
Company to Host Conference Call and Webcast Thursday, October 8th at 8:00 AM EDT/ 5:00 AM PDT

SAN DIEGO, Oct. 8, 2015 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) announced today that NBI-98854, a highly selective small molecule VMAT2 inhibitor, showed a statistically significant reduction in tardive dyskinesia during the six weeks of placebo-controlled treatment in the Kinect 3 clinical trial. This Phase III trial included moderate to severe tardive dyskinesia patients with underlying schizophrenia, schizoaffective disorder, bipolar or major depressive disorder.

The pre-specified primary efficacy endpoint was the change-from-baseline in the Abnormal Involuntary Movement Scale (AIMS) at Week 6 in the 80mg once-daily dosing group compared to placebo as assessed by central blinded video raters. The AIMS ratings at Week 6 for the 80mg once-daily NBI-98854 intention-to-treat (ITT) population was reduced 3.1 points (Least-Squares Mean) more than placebo (p<0.0001).

"We are very pleased with the outstanding efficacy and side effect profile demonstrated by NBI-98854 in the Kinect 3 study. The efficacy data from this pivotal Phase III study completes our placebo-controlled dataset for NBI-98854 in tardive dyskinesia," said Kevin C. Gorman, President and Chief Executive Officer of Neurocrine. "We will now turn our focus to completing the open-label safety portion of the studies in tardive dyskinesia patients and compiling the data for both doses of NBI-98854 to be included in the New Drug Application we intend to file with the FDA in 2016."

"The results of this Kinect 3 study demonstrate the potential of NBI-98854 to be a safe and effective treatment for patients suffering from the debilitating effects of tardive dyskinesia and we look forward to sharing additional details of this important study at upcoming scientific meetings starting in mid-2016," said Christopher F. O'Brien, Chief Medical Officer of Neurocrine. "We want to thank the trial participants and investigators who contributed to this successful placebo-controlled portion of the Kinect 3 study and we look forward to continuing our work with them in the open-label safety assessment of NBI-98854 in patients suffering from tardive dyskinesia, as well as completing the initial Tourette syndrome study later this year."

In addition to the primary efficacy endpoint, the AIMS rating for the 40mg once-daily dose and the Clinical Global Impression of Change (CGI-TD) for both doses were also evaluated. The table below summarizes the results of the AIMS ratings and CGI-TD at Week 6 for both the ITT population and a preliminary pre-specified per-protocol (PP) population. The PP population excluded subjects whose plasma concentrations of NBI-98854 were below the lower limit of quantitation (i.e., not detectable). Given the timing of plasma samples collections and the pharmacokinetic profile of NBI-98854, it was determined that these subjects had not ingested the study drug.

	Week 6			
	40mg qd	**p-value***	**80mg qd**	**p-value***
AIMS Difference from Placebo				
Least-Squares Mean (ITT population)	-1.8	0.0021	-3.1	<0.0001
Least-Squares Mean (PP population)	-2.1	0.0009	-3.6	<0.0001
CGI-TD Difference from Placebo				
Least-Squares Mean (ITT population)	-0.3	0.0742	-0.3	0.0560
Least-Squares Mean (PP population)	-0.4	0.0097	-0.4	0.0122

* Assessment of the significance of p-values based on pre-specified, fixed-sequence testing procedure

Safety Profile

During the six-week placebo-controlled treatment period NBI-98854 was generally well tolerated. The frequency of adverse events was similar among all treatment groups and treatment emergent adverse effects were consistent with those of prior studies.

Participants were also assessed utilizing the Barnes Akathisia Ratings Scale (BARS) for akathisia and the Simpson-Angus Scale (SAS) for parkinsonism. Both of these scales documented minimal symptoms at baseline and there was no worsening during the six weeks of treatment. Clinical hematology, chemistry and ECG monitoring indicated no emergent safety signals.

There were no drug-drug interactions identified in subjects who were utilizing a wide range of psychotropic and other concomitant medications.

Kinect 3 Study Design

The Kinect 3 study (ClinicalTrials.gov Identifier NCT02274558) is a randomized, parallel-group, double-blind, placebo-controlled, Phase III clinical trial utilizing the capsule formulation of NBI-98854 in moderate to severe tardive dyskinesia patients with underlying schizophrenia, schizoaffective disorder or mood disorder (including bipolar disorder or major depressive disorder). The Kinect 3 study randomized 234 subjects to either placebo, once-daily 40mg of NBI-98854 or once-daily 80mg of NBI-98854 for six weeks. Subsequent to the completion of the six week placebo-controlled dosing, all subjects are placed on once-daily 40mg or once-daily 80mg of NBI-98854 through Week 48.

The Kinect 3 study, along with the previous efficacy studies of NBI-98854, is designed to complete the placebo-controlled clinical efficacy evaluation of NBI-98854 in tardive dyskinesia. In addition to Kinect 3, a separate one-year open-label safety study of NBI-98854, Kinect 4, has also been initiated to support the anticipated 2016 filing of a New Drug Application in tardive dyskinesia.

About Tardive Dyskinesia

Tardive dyskinesia is characterized by involuntary, repetitive movements of the extremities: lip smacking, grimacing, tongue protrusion, facial movements or blinking, puckering and pursing of the lips, or involuntary movements of the limbs. These symptoms are rarely reversible and there are currently no approved treatments.

About NBI-98854

VMAT2 is a protein concentrated in the human brain that is primarily responsible for re-packaging and transporting monoamines (dopamine, norepinephrine, serotonin, and histamine) in pre-synaptic neurons. NBI-98854, developed in the Neurocrine laboratories, is a novel, highly-selective VMAT2 inhibitor that modulates dopamine release during nerve communication, while at the same time having minimal impact on the other monoamines, thereby reducing the likelihood of "off-target" side effects. NBI-98854 is designed to provide low, sustained, plasma and brain concentrations of active drug to minimize side effects associated with excessive monoamine depletion.

Modulation of neuronal dopamine levels in diseases such as tardive dyskinesia, Tourette syndrome, Huntington's chorea, schizophrenia, and tardive dystonia, which are characterized, in part, by a hyperdopaminergic state, should provide symptomatic benefits for patients with these diseases.

As announced previously, Neurocrine has also received Breakthrough Therapy Designation from the FDA for NBI-98854 in the treatment of tardive dyskinesia.

The Company is also exploring NBI-98854 in an initial Tourette syndrome clinical trial, the T-Force study. This study is an open-label, multi-dose, two-week evaluation of up to 36 subjects with Tourette syndrome. Children and adolescents enrolled in the trial are receiving a once-daily dose of NBI-98854 during a two-week treatment period to assess both the safety and tolerability of NBI-98854. Additionally, the Yale Global Tic Severity Scale and the Premonitory Urge for Tics Scale are being utilized during the study to assess the impact of NBI-98854 on the patients' Tourette symptoms. Data read out from the T-Force study is expected later in 2015.

Conference Call and Webcast Today at 8:00AM Eastern Time

Neurocrine will hold a live conference call and webcast today at 8:00 a.m. Eastern Time (5:00 a.m. Pacific Time). Participants can access the live conference call by dialing 866-952-1906 (US) or 785-424-1825 (International) using the conference ID: NBIX. The call can also be accessed via the webcast through the Company's website at http://www.neurocrine.com.

If you are unable to attend the webcast and would like further information on this announcement please contact the Investor Relations Department at Neurocrine Biosciences at (858) 617-7600. A replay of the conference call will be available approximately one hour after the conclusion of the call by dialing 800-839-1247(US) or 402-220-0470 (International) using the conference ID: NBIX. The call will be archived for one month.

Neurocrine Biosciences, Inc. discovers and develops innovative and life-changing pharmaceuticals, in diseases with high unmet medical needs, through its novel R&D platform, focused on neurological and endocrine based diseases and disorders. The Company's two lead late-stage clinical programs are elagolix, a gonadotropin-releasing hormone antagonist for women's health that is partnered with AbbVie Inc., and NBI-98854, a vesicular monoamine transporter 2 inhibitor for the treatment of movement disorders. Neurocrine intends to maintain certain commercial rights to its VMAT2 inhibitor for evolution into a fully-integrated pharmaceutical company.

Neurocrine Biosciences, Inc. news releases are available through the Company's website via the internet at http://www.neurocrine.com.

In addition to historical facts, this press release may contain 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 Neurocrine's business and finances in general, as well as risks and uncertainties associated with NBI-98854 development. Specifically, the risks and uncertainties the Company faces include risks that NBI-98854 development activities may not be completed on time or at all; risks that NBI-98854 development activities may be delayed for regulatory or other reasons, may not be successful or replicate previous clinical trial results, may fail to demonstrate that NBI-98854 is safe and effective, or may not be predictive of real-world results or of results in subsequent clinical trials; risks that NBI-98854 regulatory submissions may not occur or be submitted in a timely manner; risks that NBI-98854 may not obtain regulatory approval or that the U.S. Food and Drug Administration or regulatory authorities outside the U.S. may make adverse decisions regarding NBI-98854; risks that NBI-98854 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 NBI-98854; risks that the Company will be unable to raise additional funding, if required, to complete development of NBI-98854; risk and uncertainties relating to competitive products and technological changes that may limit demand for NBI-98854; and other risks described in the Company's quarterly report on Form 10-Q for the quarter ended June 30, 2015. Neurocrine disclaims any obligation to update the statements contained in this press release after the date hereof.

Neurocrine Biosciences, Investor Relations, (858) 617-7600