Neurocrine Announces Phase II Results of VMAT2 Inhibitor NBI-98854 for Treatment of Tardive Dyskinesia

March 26, 2012

COMPANY TO HOST CONFERENCE CALL AND WEBCAST TUESDAY, MARCH 27TH AT 8:00AM ET / 5:00AM PT

SAN DIEGO, March 26, 2012 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) today announced efficacy and safety results from a Phase II trial of NBI-98854 in 37 tardive dyskinesia patients. For the final analysis, data from one site was removed due to the inconsistent and incorrect application of the efficacy assessment protocol. With this site removed, the results showed a significant reduction in tardive dyskinesia symptoms at end of two weeks of active treatment with 50mg once-daily doses of NBI-98854.

"This Phase II trial was extremely informative. NBI-98854 displayed the efficacy and safety data we expected to see in both the 12.5mg and 50mg doses," said Chris O'Brien, Chief Medical Officer of Neurocrine Biosciences. "While we had data inconsistencies at one site, this study has provided us with the necessary information and confidence to move forward into the larger Phase IIb trials as planned."

After database lock and unblinding of study data, the pre-specified statistical assessment was conducted and the normal quality control evaluation of the output performed. An inconsistent pattern of Abnormal Involuntary Movement Scale (AIMS) scores emerged at one of the eight sites that was not evident during the blinded data review. Potential errors in randomization and drug exposure as causes for this data inconsistency were ruled out. However, a review of videotaped AIMS assessments at this single site noted discrepancies between the clinical score and the video record which were well outside of the variability associated with the AIMS. Additionally, videotaped AIMS assessments were reviewed at other sites and found to be administered appropriately.

Based on these findings, the AIMS data from this single site was removed and the statistical assessment was repeated. This post-hoc analysis demonstrated a clinically meaningful and statistically significant improvement in tardive dyskinesia symptoms for the subjects while receiving the 50mg once-daily dose. These subjects had a significant reduction in tardive dyskinesia symptoms at the end of two weeks of active treatment vs. the end of two weeks of placebo (difference in LS mean of 4.2 for the 50mg period vs. the placebo period, p-value=0.002). As expected, the 12.5mg dosing group was not statistically better during the active treatment period than during the placebo period (difference in LS mean of 0.4 for the 12.5mg period vs. placebo period, p-value=0.68).

The improvement in symptomology is also evidenced by the significant improvement in AIMS scores over baseline levels relative to placebo, excluding the one site. NBI-98854 reduced the average baseline AIMS score by 9.2 points in the 50mg period (p-value=0.0004) vs. a reduction of 4.9 points in the 12.5mg period and 4.7 for the placebo periods. A responder analysis also showed improvement in both the investigator reported Clinical Global Impression-Tardive Dyskinesia and the patient reported Patient Global Impression of Change.

When including the data from the site in question, this study did not meet the pre-specified primary endpoint of reducing the AIMS scores during active treatment periods. The efficacy results from the entire study population showed a non-significant reduction in tardive dyskinesia at the end of two weeks of active treatment vs. the end of two weeks of placebo (difference in LS mean of 1.1 for the 50mg period vs. the placebo period (n=15), p-value=0.42) (difference in LS mean of 0.7 for the 12.5mg period vs. placebo period (n=17), p-value=0.59).

The tables below summarize the primary endpoint as well as the responder analyses for all clinical sites as well as the post-hoc analysis.

	All Clinical Trial Sites			
	Baseline (mean)	**Placebo**	**12.5mg**	**50mg**
Abnormal Involuntary Movement Scale (LS Means)	14.7	9.9	9.1	8.8
			p=0.59	p=0.42
Responder Analysis				
"Much Improved or Very Much Improved"				
Clinical Global Impression-Tardive Dyskinesia	n/a	52%	65%	60%
Patient Global Impression of Change	n/a	39%	53%	60%

	Excluding Single Site			
	Baseline (mean)	Placebo	12.5mg	50mg
Abnormal Involuntary Movement Scale (LS Means)	14.9	10.3	9.9	6.1
			p=0.68	p=0.002
Responder Analysis end of Treatment				
"Much Improved or Very Much Improved"				
Clinical Global Impression-Tardive Dyskinesia	n/a	46%	67%	80%
Patient Global Impression of Change	n/a	38%	62%	80%

Safety Profile

NBI-98854 was generally safe and well tolerated; the frequency of treatment-emergent adverse events was 17% during the placebo period and 24% and 32% in the 12.5mg and 50mg treatment periods, respectively. There were no serious adverse events during the treatment period. The most common adverse event was headache and one subject in the 50mg group discontinued due to akathisia. The underlying psychiatric state of subjects was monitored using the Brief Psychiatric Ratings Scale (BPRS) and shown to be stable or improved across study groups declining from 32 at baseline to 28 at the end of the study. There were no drug-drug interactions identified in subjects who were utilizing a range of psychotropic and other concomitant medications.

"While not ideal, this study served its primary purpose of informing the larger Phase IIb studies. The 50mg once-daily dose of NBI-98854 provided tardive dyskinesia sufferers with a remarkable improvement of symptoms, coupled with an excellent safety and tolerability profile," said Kevin C. Gorman President and Chief Executive Officer of Neurocrine Biosciences. "We will apply additional controls in future studies to ensure appropriate scoring of AIMS."

Trial Design

This trial was a randomized, double-blind, placebo controlled, cross-over, Phase II clinical trial utilizing NBI-98854 in tardive dyskinesia patients at eight investigator sites. This 37 subject study assessed once-daily NBI-98854 (12.5mg and 50mg) over a two week dosing period. The primary endpoint of the study was a comparison of placebo vs. active scores utilizing the Abnormal Involuntary Movement Scale (AIMS).

Next Steps for NBI-98854

A placebo controlled, double-blind, parallel design, multiple dose, twelve week Phase IIb study is planned to assess six-week dosing of NBI-98854 against placebo, followed by six weeks of active treatment with NBI-98854. The study will incorporate a capsule formulation of NBI-98854 and will be initiated in mid-2012, with top-line data anticipated by year-end.

About the Abnormal Involuntary Movement Scale (AIMS)

The AIMS was developed in 1976 and has been used extensively in movement disorder assessments. It consists of distinct ratings of regional involuntary body movements that are observed during a structured neurological examination. Items one through seven rate facial, extremity and trunk movements; items eight through ten are overall global judgments of severity, incapacitation and patient awareness. All ten items are scored on a zero to four scale with zero being rated as none, and four being rated as severe. The primary endpoint is assessed on items one through seven.

About Tardive Dyskinesia

Tardive dyskinesia is characterized by involuntary, repetitive movements of the extremities, lip smacking, grimacing, tongue protrusion, rapid eye movements or blinking, puckering and pursing of the lips, or impaired movement of the fingers. These symptoms are rarely reversible and there is currently no known treatment.

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) among nerve cells. 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 dopamine depletion. The Company has completed three-month in vivo toxicology studies to support longer dosing regimens.

NBI-98854 may also be useful in other disorders such as Huntington's chorea, schizophrenia, Tourette's syndrome, and tardive dystonia.

Conference Call and Webcast Information

The Company will host a live conference call and webcast to provide additional details of this study tomorrow morning, Tuesday March 27, 2012 at 8:00 a.m. Eastern Time (5:00 a.m. Pacific Time). Participants can access the live conference call by dialing 1-800-895-0198 (US) or 785-424-1053 (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. Slides will also be made available through www.neurocrine.com for the conference call and webcast.

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 1-800-283-8520 (US) or 402-220-0870 (International) using the conference ID: NBIX. The call will be archived for three weeks.

About Neurocrine Biosciences

Neurocrine Biosciences, Inc. is a biopharmaceutical company focused on neurological and endocrine diseases and disorders. Our product candidates address some of the largest pharmaceutical markets in the world, including endometriosis, stress-related disorders, pain, tardive dyskinesia, uterine fibroids, diabetes, insomnia, and other neurological and endocrine-related diseases and disorders. 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 the Company's VMAT2 program and Company overall. Specifically, the risks and uncertainties the Company faces with respect to the Company's VMAT2 program include, but are not limited to; risk that NBI-98854 will not proceed to later stage clinical trials and risk that the Company's clinical trials will fail to demonstrate that NBI-98854 is safe and effective. With respect to its pipeline overall, the Company faces risk that it will be unable to raise additional funding required to complete development of all of its product candidates; risk relating to the Company's dependence on contract manufacturers for clinical drug supply; risks associated with the Company's dependence on corporate partners for development, commercial manufacturing and marketing and sales activities for the Company's partnered programs; uncertainties relating to patent protection and intellectual property rights of third parties; risks and uncertainties relating to competitive products and technological changes that may limit demand for the Company's products; and the other risks described in the Company's report on Form 10-K for the year ended December 31, 2011. Neurocrine undertakes no obligation to update the statements contained in this press release after the date hereof.

SOURCE Neurocrine Biosciences, Inc.

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