Neurocrine Biosciences Announces Successful Phase IIa Clinical Trial for VMAT2 Inhibitor

April 5, 2011

SAN DIEGO, April 5, 2011 /PRNewswire via COMTEX/ --

Neurocrine Biosciences, Inc. (NASDAQ: NBIX) announced today that it has completed the dosing and preliminary assessment of the initial cohort of Tardive Dyskinesia patients using its proprietary Vesicular Monoamine Transporter 2 inhibitor (VMAT2), NBI-98854. Based on this data, the Company is initiating the Investigational New Drug (IND) application process with the U.S. Food and Drug Administration (FDA).

"We are very pleased with these preliminary results from our VMAT2 Phase IIa study," said Christopher F. O'Brien, Chief Medical Officer of Neurocrine Biosciences. "Over the twelve days of treatment with our VMAT2 inhibitor, subjects showed a marked improvement in abnormal hyperkinetic movements. The drug was generally well tolerated and showed the desired pharmacokinetic profile previously demonstrated in two Phase I studies."

The open-label Phase IIa study was designed to assess efficacy, safety and tolerability of NBI-98854 in up to ten schizophrenia patients who have moderate to severe Tardive Dyskinesia over a twelve-day period. The impact on the dyskinesia was assessed utilizing the Abnormal Involuntary Movement Scale (AIMS). The dosing regimen consisted of three, four-day periods of NBI-98854 at increasing doses of 12.5mg, 25mg and 50mg administered once daily. After discontinuation of NBI-98854, a seven-day washout period was followed by a final assessment. The study inclusion criteria included a baseline total score of at least nine on the first seven components of the AIMS, with at least two body regions receiving scores of moderate (3) or severe (4). For this cohort of six subjects, the mean baseline score was 14.3 (AIMS total items 1-7, possible total score of 28).

After the twelve days of dosing in six subjects, the mean AIMS score decreased to 8.4, a reduction of 41.3%. Reduction in abnormal involuntary movements was shown across multiple assessment points. After the seven-day washout period most patients' AIMS scores returned to their baseline levels. The adverse events reported during administration of study drug were transient and mild or moderate including one subject with dizziness and one with restlessness. One subject became anxious and agitated seven days after stopping the study medication due to return of baseline-intensity dyskinesia.

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 ten distinct ratings that are observed both spontaneously and upon activation. Ratings one through seven score facial, extremity and trunk movements; items eight through ten are overall global judgments of severity, incapacitation and patient awareness. All ten ratings are scored on a zero to four scale with zero being rated as none, and four being rated as severe.

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.

NBI-98854 may well be useful in other disorders such as Huntington's chorea, schizophrenia, Tourette's syndrome, and Tardive Dystonia.

Next Steps for NBI-98854

All of the clinical work to date for NBI-98854 has been conducted in Canada. The Company plans to open an IND in the United States this year. The current Phase IIa study has the option of enrolling up to four additional subjects over the next few months. While additional data are not necessary to proceed to IND filing in the US, these data would be considered informative to the overall development program. The Company will be conducting three-month in vivo toxicology studies to support longer dosing regimens. A placebo-controlled cross-over design Phase II study is expected to be initiated in the United States during the third quarter of this year to further assess NBI-98854. A larger, longer term Phase IIb study is planned to be initiated in early 2012 to assess three-month dosing of NBI-98854.

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 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, 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, 2010. Neurocrine undertakes no obligation to update the statements contained in this press release after the date hereof.

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