Neurocrine Biosciences Receives Breakthrough Therapy Designation for NBI-98854 in Tardive Dyskinesia

October 30, 2014

SAN DIEGO, Oct. 30, 2014 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) announced today that the U.S. Food and Drug Administration (FDA) has granted Breakthrough Therapy Designation for its Vesicular Monoamine Transporter 2 inhibitor, NBI-98854, in tardive dyskinesia. A breakthrough therapy designation is granted for a drug that is intended to treat a serious or life-threatening disease or condition and preliminary clinical evidence indicates that the drug may demonstrate substantial improvement on clinically significant endpoints over available therapies. The breakthrough designation also allows intensive discussions with the FDA which are intended to expedite the development and review process of eligible drugs.

"We are pleased that after reviewing our dataset the FDA recognizes that NBI-98854 is potentially an important therapy for the treatment of tardive dyskinesia, and we look forward to working closely with the Division of Psychiatry to advance our development program," said Christopher F. O'Brien, Chief Medical Officer of Neurocrine Biosciences.

The Breakthrough Therapy Designation was granted, in part, based on the results of the Phase IIb Kinect studies of NBI-98854 in approximately 220 patients with tardive dyskinesia. Data from the Phase IIb program were presented in June 2014 at the Annual Congress of Parkinson's Disease and Movement Disorders in Stockholm, Sweden.

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.

The Company recently initiated a Phase III study of NBI-98854 in tardive dyskinesia, the Kinect 3 study. 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 for tardive dyskinesia. The Company also intends to conduct a separate one-year open-label safety study of NBI-98854 to support the anticipated 2016 filing of a New Drug Application with the FDA in tardive dyskinesia.

In addition to the tardive dyskinesia clinical effort, the Company has recently initiated a clinical study assessing NBI-98854 in children and adolescents with Tourette syndrome.

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 Neurocrine Biosciences

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 a wholly owned 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 the Company's VMAT2 program and the 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 the Company's VMAT2 Phase III clinical program will fail to demonstrate that NBI-98854 is safe and effective; risk that the VMAT2 Phase III program will be delayed for regulatory or other reasons; risk that the Breakthrough Therapy designation may not result in an expedited development and review process for NBI-98854 and may not lead to regulatory approval; and risk that the Company will not be able to submit an NDA filing for NBI-98854 tardive dyskinesia as planned; and risk that the Company will be unable to complete the Tourette syndrome Phase I clinical trial for regulatory or other reasons. With respect to its business 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 contractors for clinical drug supply, commercial manufacturing and marketing and sales activities; uncertainties relating to patent protection and intellectual property rights of third parties; 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; risks and uncertainties relating to competitive products and technological changes that may limit demand for the Company's products if approved. The Company also faces the other risks described in the Company's annual report on Form 10-K for the year ended December 31, 2013 and quarterly reports on Form 10-Q for the quarters ended March 31, 2014 and June 30, 2014. 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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