Neurocrine Announces Expansion Of VMAT2 Inhibitor Program With Initiation Of Tourette Syndrome Clinical Study

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NBI-98854 TO BE EVALUATED FOR BOTH CHILDREN AND ADOLESCENTS IN T-FORCE CLINICAL TRIAL

SAN DIEGO, Oct. 2, 2014 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) announced today that it has initiated a clinical trial of NBI-98854, a proprietary small molecule Vesicular Monoamine Transporter 2 (VMAT2) inhibitor, in both children and adolescents with Tourette syndrome.

The T-Force study is an open-label, multi-dose, two-week study of 36 subjects with Tourette syndrome. Children and adolescents will receive once-daily dosing of NBI-98854 during a two-week treatment period to assess both the safety and tolerability of NBI-98854 in Tourette patients. Additionally, the Yale Global Tic Severity Scale and the Premonitory Urge for Tics Scale will be employed during the study to assess the impact of NBI-98854 on the patients' Tourette symptoms. Data readout from this study is expected in 2015.

"Advancing NBI-98854 into clinical evaluation of Tourette syndrome represents another significant achievement for our VMAT2 franchise," said Kevin C. Gorman, President and Chief Executive Officer of Neurocrine Biosciences. "An important aspect of this initial Tourette syndrome trial is that we are exploring NBI-98854 in children age six to eleven, the target age range for therapy."

T-Force Study Design

The T-Force study is an open-label, multiple ascending dose, pharmacokinetic and pharmacodynamic, study to evaluate the safety, tolerability and exposure-response of NBI-98854 in children and adolescents with Tourette syndrome. A total of 36 patients will be evaluated over 14 days of once-daily dosing followed by 7 days off-drug at approximately 10 study centers in the United States. The study will be divided into two dosing groups consisting of children (ages 6-11) and adolescents (ages 12-18), and each age group will be further divided into three dosing cohorts of six patients each. After completing the initial two weeks of dosing with the first adolescent cohort, an independent review of both safety and pharmacokinetic results will occur prior to escalating the dose level for the second cohort of adolescents. In parallel, while initiating the second cohort of adolescents, the first cohort of children (ages 6-11) will also be administered NBI-98854 for a two-week period. Subsequent dose escalations for children and adolescents will be based, in part, on the pharmacokinetic and safety data from the previous cohort in each age group. Additionally, the patient's Tourette symptoms will be evaluated weekly via the Yale Global Tic Severity Scale, the Premonitory Urge for Tics Scale as well as an overall Clinical Global Impression in Tourette syndrome Scale.

About Tourette Syndrome

Tourette syndrome is a neurological disorder that consists of rapid, non-rhythmic stereotyped motor and vocal tics. Motor tics are typically characterized by facial grimacing, head jerks, extremity movements and other dystonic movements. Vocal tics typically include grunting, throat clearing, and repeating words and phrases. The average age of onset for Tourette syndrome is at six years, with symptoms reaching their peak severity at approximately age ten. Tourette syndrome is more commonly diagnosed in males than females and may be associated with attention deficit hyperactivity disorder and obsessive compulsive disorder. There are approximately 400,000 people with Tourette syndrome in the United States.

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.

In addition to this Tourette syndrome study, the Company will initiate the Phase III pivotal study assessing NBI-98854 in tardive dyskinesia during the fourth quarter of 2014.

The Company has two distinct Investigational New Drug Applications, tardive dyskinesia and Tourette syndrome, open with the Division of Psychiatry Products at the FDA.

About Neurocrine Biosciences

Neurocrine Biosciences, Inc. is a clinical stage drug discovery company primarily focused on neurological and endocrine based diseases and disorders. The Company discovers and develops innovative pharmaceuticals, in diseases with high unmet medical needs or where the existing drug classes are inadequate, through a disciplined yet entrepreneurial process. Utilizing a portfolio approach to drug discovery, Neurocrine has multiple small molecule drug candidates at various stages of pharmaceutical development. Neurocrine's two lead late stage clinical programs are elagolix, a GnRH antagonist for women's health that is partnered with AbbVie Inc., and a wholly owned VMAT2 inhibitor for the treatment of movement 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 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 program in tardive dyskinesia will be delayed for regulatory or other reasons; risk that the guidance provided by the FDA in the End-of-Phase II meeting may be modified or may not lead to regulatory approval; risk that the Company will be unable to complete the T-Force clinical trial in Tourette syndrome for regulatory or other reasons; risk that NBI-98854 will not proceed to later stage clinical trials in Tourette syndrome; and risk that the Company's Phase III or other clinical trials will fail to demonstrate that NBI-98854 is safe and effective. 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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