Neurocrine Announces Phase II Results of VMAT2 Inhibitor INGREZZA® For Treatment of Tourette Syndrome

May 23, 2017

Study Provides Clear Path Forward for Future Pivotal Studies
Company to Host Conference Call and Webcast Tuesday, May 23rd at 5:00 P.M. ET / 2:00 P.M. PT

SAN DIEGO, May 23, 2017 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) announced today that the initial Phase II Tourette syndrome T-Force GREEN study of INGREZZA®(valbenazine), a small molecule VMAT2 inhibitor, did not meet its primary endpoint. The pre-specified primary endpoint was the change-from-baseline between the placebo and active groups in the Yale Global Tic Severity Scale (YGTSS) at Week 6 in the intent-to-treat (ITT) population.

Exposure-response analysis showed that the selected doses for this placebo-controlled Phase II study were below the therapeutic range for adequate tic reduction in the majority of pediatric subjects. For the subset of subjects with pharmaceutical exposure in the appropriate range, there was a substantial reduction in tics (e.g., -11.3 to -13.7 points on the YGTSS). For subjects with sub-therapeutic exposure, tic reduction was comparable to placebo (e.g., -4.7 to -8.3 points on the YGTSS). In this study, adverse events were consistent with those observed in previous INGREZZA studies. There were a total of four discontinuations due to adverse events, two in each of the placebo and INGREZZA arms.

"This study showed that we underestimated the INGREZZA dose needed for the pediatric population but also provided us with a clear-cut view into the level of dosing required for future studies," said Christopher F. O'Brien, Chief Medical Officer of Neurocrine. "We have developed a complete exposure-response model from this study that we believe accurately defines the appropriate dose-range to be tested in the next clinical study that will be started later this year. We were very pleased with the conduct of the T-Force GREEN study in that we were able to identify the appropriate pediatric subjects for the clinical trial, and the investigators demonstrated consistent and appropriate application of the Yale Global Tic Severity Scale."

About T-Force GREEN

The T-Force GREEN study was a randomized, double-blind, placebo-controlled, multi-dose, parallel group, Phase II study of 98 children and adolescents. Pediatric Tourette syndrome patients received once-daily dosing of INGREZZA or placebo during a six-week treatment period to assess the safety, tolerability and efficacy of INGREZZA. The primary endpoint of this study was the change-from-baseline of the Yale Global Tic Severity Scale between placebo and active treatment groups at the end of week six. The study assessed two doses of INGREZZA for each of the child and adolescent study arms.

Additionally, the Company is also conducting an open-label, fixed-dose study of INGREZZA in up to 180 subjects with Tourette syndrome. This study has recently completed enrollment and was designed to enroll up to 90 children and adolescents and up to 90 adults who have completed either of the two placebo-controlled Tourette syndrome clinical trials: T-Force GREEN or T-Forward. This Phase II study will assess the long-term safety and tolerability of INGREZZA in children and adults with Tourette syndrome.

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 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.

About INGREZZA

INGREZZA, a selective VMAT2 inhibitor, is the first and only product approved for the treatment of adults with tardive dyskinesia (TD). INGREZZA inhibits VMAT2 and is thought to work by reducing the amount of dopamine released in a region of the brain that controls movement and motor function, helping to regulate nerve signaling in adults with TD. VMAT2 is a protein in the brain that packages neurotransmitters, such as dopamine, for transport and release in presynaptic neurons. INGREZZA, developed in Neurocrine's laboratories, is novel in that it selectively inhibits VMAT2 with no appreciable binding affinity for VMAT1, dopaminergic (including D2), serotonergic, adrenergic, histaminergic, or muscarinic receptors. Additionally, INGREZZA can be taken once-daily, and together with psychiatric medications such as antipsychotics or antidepressants.

Conference Call and Webcast Today at 5:00PM Eastern Time

Neurocrine will hold a live conference call and webcast today at 5:00 p.m. Eastern Time (2:00 p.m. Pacific Time). Participants can access the live conference call by dialing 877-876-9170 (US) or 785-424-1672 (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.

About Neurocrine Biosciences

Neurocrine Biosciences is a San Diego based biotechnology company focused on neurologic, psychiatric and endocrine related disorders. In April of 2017, the FDA approved INGREZZA (valbenazine) capsules for the treatment of adults with tardive dyskinesia (TD). INGREZZA is a novel, selective vesicular monoamine transporter 2 (VMAT2) inhibitor, and is the first and only FDA-approved product indicated for the treatment of adults with TD. The Company markets INGREZZA in the United States. The Company's three late-stage clinical programs are: elagolix, a gonadotropin-releasing hormone antagonist for women's health that is partnered with AbbVie Inc.; opicapone, a novel, once-daily, peripherally-acting, highly-selective catechol-o-methyltransferase inhibitor under investigation as adjunct therapy to levodopa in Parkinson's patients; and INGREZZA (valbenazine), a

novel, once-daily, selective VMAT2 inhibitor under investigation for the treatment of Tourette syndrome.

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 INGREZZA (valbenazine) development for Tourette syndrome. Specifically, the risks and uncertainties the Company faces include risks that INGREZZA development activities may not be completed on time or at all; risks that INGREZZA development activities may not be completed or may be delayed for regulatory or other reasons, may not be successful or replicate previous clinical trial results, may fail to demonstrate that INGREZZA is safe, tolerable or effective in the Tourette syndrome population, or may not be predictive of real-world results or of results in subsequent clinical trials; risks that regulatory submissions may not occur or be submitted in a timely manner; risks that INGREZZA may not obtain regulatory approval for Tourette syndrome, or that the U.S. Food and Drug Administration or regulatory authorities outside the U.S. may make adverse decisions regarding INGREZZA; risks that INGREZZA 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 INGREZZA; risks and uncertainties relating to competitive products and technological changes that may limit demand for INGREZZA; and other risks described in the Company's quarterly report on Form 10-Q for the quarter ended March 31, 2017. Neurocrine disclaims any obligation to update the statements contained in this press release after the date hereof.

SOURCE Neurocrine Biosciences, Inc.

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