Neurocrine Granted FDA Orphan Drug Designation for Valbenazine for the Treatment of Pediatric Patients With Tourette Syndrome

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SAN DIEGO, Oct. 23, 2017 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) today announced that valbenazine, a novel selective vesicular monoamine transporter 2 (VMAT2), has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) for the treatment of pediatric patients with Tourette syndrome. Tourette syndrome is a neurological disorder that becomes evident in early childhood or adolescence and is characterized by motor and vocal tics. Valbenazine is marketed in the United States as INGREZZA® (valbenazine) capsules for the treatment of adults with tardive dyskinesia.

"We are pleased that the FDA has granted valbenazine orphan drug designation to treat pediatric patients with Tourette syndrome as it represents another significant regulatory milestone for our company," said Malcolm Lloyd-Smith, Chief Regulatory Officer at Neurocrine Biosciences. "We will continue our clinical development program for Tourette's focused on pediatric patients as the first symptoms of Tourette syndrome typically present early in childhood and represent a significant challenge for these young patients."

Orphan drug designation is granted by the FDA to drugs that are intended to treat rare diseases or conditions for patients in the U.S. The orphan drug designation allows the orphan drug indication for the drug to be eligible for a seven-year period of U.S. marketing exclusivity upon approval of the drug, as well as other development assistance and financial incentives.

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 stereotyped 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® (valbenazine) capsules
INGREZZA, a selective VMAT2 inhibitor, is the first FDA approved product indicated for the treatment of adults with tardive dyskinesia, a condition associated with uncontrollable, abnormal and repetitive movements of the trunk, extremities and/or face.

INGREZZA 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 tardive dyskinesia. 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 as one capsule, once-daily, together with psychiatric medications such as antipsychotics or antidepressants. INGREZZA is currently in clinical development for the treatment of Tourette syndrome.

Important Safety Information
Warnings & Precautions
Somnolence
INGREZZA can cause somnolence. Patients should not perform activities requiring mental alertness such as operating a motor vehicle or operating hazardous machinery until they know how they will be affected by INGREZZA.

QT Prolongation
INGREZZA may prolong the QT interval, although the degree of QT prolongation is not clinically significant at concentrations expected with recommended dosing. INGREZZA should be avoided in patients with congenital long QT syndrome or with arrhythmias associated with a prolonged QT interval. For patients at increased risk of a prolonged QT interval, assess the QT interval before increasing the dosage.

Adverse Reactions
The most common adverse reaction (≥5% and twice the rate of placebo) is somnolence. Other adverse reactions (≥2% and >placebo) include: anticholinergic effects, balance disorders/falls, headache, akathisia, vomiting, nausea, and arthralgia.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit MedWatch at www.fda.gov/medwatch or call 1-800-FDA-1088.

Please see INGREZZA full Prescribing Information at www.INGREZZA.com/HCP

About Neurocrine Biosciences
Neurocrine Biosciences is a San Diego based biotechnology company focused on neurologic, psychiatric and endocrine related disorders. The Company markets INGREZZA® (valbenazine) capsules in the United States for the treatment of adults with tardive dyskinesia. INGREZZA is a novel, selective vesicular monoamine transporter 2 (VMAT2) inhibitor, and is the first FDA approved product indicated for the treatment of adults with tardive dyskinesia. 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, 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.

Forward Looking Statements
In addition to historical facts, this press release contains forward-looking statements that involve a number of risks and uncertainties. These statements include, but are not limited to, statements related to the benefits to be derived from INGREZZA and whether results from INGREZZA's clinical trials are indicative of real-world results. 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; risks that the Company may not complete its development program in Tourette syndrome timely, or at all; risks and uncertainties relating to competitive products and technological changes that may limit demand for INGREZZA; risks associated with the Company's dependence on third parties for development and manufacturing activities related to INGREZZA and the ability of the Company to manage these third parties; risks that the FDA or other regulatory authorities may make adverse decisions regarding INGREZZA; risks that INGREZZA clinical trials may not be predictive of real-world results or of results in subsequent clinical trials; risks that INGREZZA may be alleged to infringe upon the proprietary rights of third parties, or have unintended side effects, adverse reactions or incidents of misuse; and other risks described in the Company's periodic reports filed with the Securities and Exchange Commission, including without limitation the Company's Quarterly Report on Form 10-Q for the quarter ended June 30, 2017. The Company disclaims any obligation to update the statements contained in this press release after the date hereof.

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