Neurocrine Biosciences to Present Data on INGREZZA® (valbenazine) for Tardive Dyskinesia; Opicapone and VY-AADC for Parkinson's Disease, at the 2019 International Congress of Parkinson's Disease and Movement Disorders®

September 17, 2019

- Phase III Data Analyses Evaluate the Long-Term Benefit of INGREZZA® (valbenazine) 40 mg and Early Response to Treatment in Patients with Tardive Dyskinesia

- Data Examine Effect of Opicapone on COMT Enzyme and Levodopa Concentration in Patients with Parkinson's Disease

- Phase 1b Data Analyze Parkinson's Disease Progression in Patients Treated with Gene Therapy, VY-AADC

SAN DIEGO, Sept.17, 2019 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) today announced it will present data analyses from its movement disorder programs, including INGREZZA® (valbenazine) capsules, the first U.S. Food and Drug Administration (FDA) approved treatment for adults with tardive dyskinesia (TD); opicapone, an investigational adjunctive treatment for Parkinson's disease (PD) in the U.S.; and VY-AADC, a gene therapy for PD acquired through a strategic collaboration with Voyager Therapeutics. The data will be presented at the 2019 International Congress of Parkinson's Disease and Movement Disorders® in Nice, France, Sept. 22-26, 2019.

"We look forward to sharing data from our movement disorder programs in tardive dyskinesia and Parkinson's disease, including long-term data in patients with tardive dyskinesia who respond early to INGREZZA treatment and new long-term data analyses of INGREZZA at the 40 mg dose. We will also present data from our Parkinson's disease programs, including data describing the pharmacokinetics and pharmacodynamic effect of opicapone on COMT enzyme activity and levodopa concentration in patients with Parkinson's disease, as well as an analysis of Parkinson's disease progression in patients treated with the gene therapy, VY-AADC," said Eiry W. Roberts, M.D., Chief Medical Officer at Neurocrine Biosciences. "These data add to our understanding of these debilitating movement disorders that can place a substantial burden on the lives of patients."

The five abstracts that will be presented at the 2019 International Congress of Parkinson's Disease and Movement Disorders are:

INGREZZA® (valbenazine)

- **Long-Term Outcomes in Patients with Tardive Dyskinesia Who Were Early Responders with Valbenazine**
  Abstract number: 1456; Topic: Drug-Induced Movement Disorders; Tuesday, Sept. 24 from 1:45-3:15 p.m. CEST
- **Long-Term Outcomes with Valbenazine 40 mg/day in Adults with Tardive Dyskinesia**
  Abstract number: 1464; Topic: Drug-Induced Movement Disorders; Tuesday, Sept. 24 from 1:45-3:15 p.m. CEST

Opicapone

- **Pharmacokinetics of Opicapone and Effect on COMT and Levodopa Pharmacokinetics in Patients with Parkinson's Disease**
  Abstract number: 143; Topic: Clinical Trials, Pharmacology and Treatment; Monday, Sept. 23 from 1:45-3:15 p.m. CEST
- **Effect of Once-Daily Opicapone on the Pharmacokinetics of Repaglinide**
  Abstract number: 144; Topic: Clinical Trials, Pharmacology and Treatment; Monday, Sept. 23 from 1:45-3:15 p.m. CEST

VY-AADC

- **Longitudinal Analysis of the Modified Hoehn and Yahr Disease Stage in PD-1101, a Phase 1b Clinical Study of VY-AADC01**
  Abstract number: 562; Topic: Gene and Cell-Based Therapies; Monday, Sept. 23 from 1:45-3:15 p.m. CEST

About Tardive Dyskinesia (TD)
Tardive dyskinesia (TD) is a movement disorder that is characterized by uncontrollable, abnormal and repetitive movements of the face, torso and/or other body parts, which may be disruptive and negatively impact patients. The condition is caused by prolonged

use of treatments that block dopamine receptors in the brain, such as antipsychotics commonly prescribed to treat mental illnesses such as schizophrenia, bipolar disorder and depression and certain anti-nausea medications. In patients with TD, these treatments are thought to result in irregular dopamine signaling in a region of the brain that controls movement. The symptoms of TD can be severe and are often persistent and irreversible. TD is estimated to affect at least 500,000 people in the U.S.

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 face, torso and/or other body parts.

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 for the treatment of tardive dyskinesia as one capsule, once-daily, together with psychiatric medications such as antipsychotics or antidepressants.

Important Safety Information

Contraindications
INGREZZA is contraindicated in patients with a history of hypersensitivity to valbenazine or any components of INGREZZA. Rash, urticaria, and reactions consistent with angioedema (e.g., swelling of the face, lips, and mouth) have been reported.

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.

Parkinsonism
INGREZZA may cause Parkinsonism in patients with tardive dyskinesia. Parkinsonism has also been observed with other VMAT2 inhibitors. Reduce the dose or discontinue INGREZZA treatment in patients who develop clinically significant parkinson-like signs or symptoms.

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/PI.

About Parkinson's Disease
Parkinson's disease is a chronic, progressive and debilitating neurodegenerative disorder that affects approximately one million people in the U.S. and six million people worldwide. Parkinson's disease is characterized by a loss of dopamine and its function. Dopamine is a chemical "messenger" that is produced in the brain and is involved in the control of movement. As Parkinson's progresses, dopamine production steadily decreases resulting in slowed movement (bradykinesia), tremor, rigidity, impaired posture and balance, and speech and writing difficulty.

There is no present cure for Parkinson's disease and management consists of controlling the motor symptoms primarily through administration of dopaminergic therapies, including levodopa. While levodopa improves patients' motor symptoms, as the disease progresses, the beneficial effects of levodopa begin to wear off more quickly, causing symptoms to worsen as patients experience motor fluctuations throughout the day.

About Opicapone
Opicapone, an investigational treatment for Parkinson's disease in the U.S., is a novel, once-daily, selective catechol-O-methyltransferase (COMT) inhibitor. Opicapone works by prolonging the clinical effect of levodopa through decreasing its conversion rate into 3-O-methyldopa to allow for greater availability in the brain. Discovered in the BIAL laboratories, it is designed to provide patients and physicians with a once-daily option as an adjunct treatment to levodopa/carbidopa for Parkinson's disease.

In June 2016, the European Commission approved ONGENTYS® (opicapone) as an adjunct therapy to preparations of

levodopa/DOPA decarboxylase inhibitors (DDCIs) in adult patients with Parkinson's disease and end-of-dose motor fluctuations who cannot be stabilized on those combinations. In February 2017, Neurocrine Biosciences entered into an exclusive licensing agreement with BIAL for the development and commercialization of opicapone in the U.S. and Canada. Opicapone is an investigational drug not approved for use in the U.S. or Canada.

About VY-AADC
Dopamine is made in the brain when the enzyme AADC (Aromatic l-amino acid decarboxylase) converts the chemical levodopa to dopamine. Levodopa, AADC, and dopamine are each present at normal levels in healthy people. As Parkinson's disease worsens, there is less AADC enzyme in parts of the brain where it is needed to convert levodopa to dopamine. When this happens, patients' motor function may worsen with a less predictable response to medications.

VY-AADC, an investigational gene therapy, is designed to put the AADC enzyme into brain cells where it can convert levodopa to dopamine. To do this, the *AADC* gene is delivered inside a transporter called "adeno-associated viral vector" (AAV). Interim results from an open-label Phase Ib trial demonstrated that administration of VY-AADC to the putamen using intraoperative monitoring with MRI facilitated targeted delivery of the investigational gene therapy with dose-dependent increases in AADC enzyme expression and improvements in clinical measures and has been well-tolerated to date.

In January 2019, Neurocrine Biosciences and Voyager Therapeutics announced a strategic collaboration focused on the development and commercialization of gene therapy programs, VY-AADC for Parkinson's disease and VY-FXN01 for Friedreich's ataxia, as well as rights to two programs to be determined.

About Neurocrine Biosciences
Neurocrine Biosciences (Nasdaq: NBIX) is a neuroscience-focused, biopharmaceutical company with more than 25 years of experience discovering and developing life-changing treatments for people with serious, challenging and under-addressed neurological, endocrine and psychiatric disorders. The company's diverse portfolio includes FDA-approved treatments for tardive dyskinesia and endometriosis* and clinical development programs in multiple therapeutic areas including Parkinson's disease, congenital adrenal hyperplasia, uterine fibroids* and polycystic ovary syndrome*. Headquartered in San Diego, Neurocrine Biosciences specializes in targeting and interrupting disease-causing mechanisms involving the interconnected pathways of the nervous and endocrine systems. For more information, visit **neurocrine.com**, and follow the company on **LinkedIn**. *(*in collaboration with AbbVie)*

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 Neurocrine's products and product candidates. 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 the commercialization of INGREZZA; risks that the opicapone NDA may not obtain regulatory approval from the FDA or such approval may be delayed or conditioned; risks or uncertainties related to the development of the Company's product candidates; risks and uncertainties relating to competitive products and technological changes that may limit demand for INGREZZA or a product candidate; risks associated with the Company's dependence on BIAL for development and manufacturing activities related to opicapone, and the ability of the Company to manage BIAL; risks that clinical development activities may not be completed on time or at all; risks that clinical development activities may be delayed for regulatory, manufacturing, or other reasons, may not be successful or replicate previous clinical trial results, may fail to demonstrate that our product candidates are safe and effective, or may not be predictive of real-world results or of results in subsequent clinical trials; risks that INGREZZA and/or our product candidates may be precluded from commercialization by the proprietary or regulatory 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, 2019. Neurocrine disclaims any obligation to update the statements contained in this press release after the date hereof.

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