



## Neurocrine Biosciences to Present Data Analyses on INGREZZA® (valbenazine) and Opicapone at the 2019 American Academy of Neurology Annual Meeting

April 30, 2019

- **Long-Term Data from Two Phase III Trials Show INGREZZA Sustained Improvement in Tardive Dyskinesia Symptoms in Patients Taking Concomitant Medications for Psychiatric Disorders**
- **Analysis of Phase III Studies Demonstrates Treatment with Opicapone Results in Improvements in ON-time Without Troublesome Dyskinesia in Parkinson's Disease Patients Treated with Levodopa**

SAN DIEGO, April 30, 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 treatment for Parkinson's disease (PD); and VY-AADC, a gene therapy for PD acquired through a strategic collaboration with Voyager Therapeutics. The data will be presented at the 2019 American Academy of Neurology (AAN) Annual Meeting in Philadelphia, May 4-10, 2019.



"Movement disorders such as Parkinson's disease and tardive dyskinesia are difficult conditions to manage and place a burden on the lives of patients," said Eiry W. Roberts, M.D., Chief Medical Officer at Neurocrine Biosciences. "We look forward to sharing long-term data analyses of INGREZZA in patients with tardive dyskinesia who are taking concomitant antipsychotic medications and data from our Parkinson's disease programs. These data are important in providing neurologists and healthcare providers with an understanding of the benefits of INGREZZA and the potential of opicapone and VY-AADC for patients suffering from movement disorders."

The oral and poster presentations that will be presented at the 2019 AAN Annual Meeting are:

### Oral Presentation Session:

- **Once-Daily Opicapone Increases ON-Time in Patients with Parkinson's Disease: Results from Two Phase 3 Studies**  
Program Number S4.003, Oral Presentation Session IV: Clinical Trials in Movement Disorders, Sunday, May 5, 1:22 p.m. ET

### Poster Presentation Sessions:

- **PD-1102: A Phase 1 Study of VY-AADC01 Administered Using a Posterior Approach in Patients with Parkinson's Disease and Motor Fluctuations (Voyager Therapeutics)**  
Program Number P1.8-017, Poster Presentation Session I: Parkinson's Disease Surgical, Stem Cell and Gene Therapies, Sunday, May 5, 5:30 p.m.-6:30 p.m. ET
- **Effects of Concomitant Medication Use on Tardive Dyskinesia Outcomes in Long-Term Valbenazine Trials**  
Program Number P4.8-035, Poster Presentation Session IV: Other Hyperkinetic Movement Disorders and Tremor, Wednesday, May 8, 5:30 p.m.-6:30 p.m. ET

### 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 vesicular monoamine transporter 2 (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.

### **Adverse Reactions**

The most common adverse reaction ( $\geq 5\%$  and twice the rate of placebo) is somnolence. Other adverse reactions ( $\geq 2\%$  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](http://www.fda.gov/medwatch) or call 1-800-FDA-1088.

**Please see INGREZZA full Prescribing Information at [www.INGREZZA.com/PI](http://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 levodopa therapies. While levodopa improves the control of Parkinson's motor symptoms, the disease progresses, and the beneficial effects of levodopa begin to wear off, symptoms worsen and patients experience motor fluctuations.

### **About Opicapone**

Opicapone, an investigational treatment for Parkinson's disease, is a novel, once-daily, peripherally-acting, selective catechol-o-methyltransferase (COMT) inhibitor. Opicapone works by prolonging the duration of effect of levodopa through decreasing its conversion rate into 3-O-methyldopa. A novel compound discovered in the BIAL – Portela & CA, S.A. (BIAL) laboratories, it is designed to provide patients and physicians with a once-daily option for the treatment of 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 United States and Canada. Opicapone is an investigational drug not approved for use in the United States or Canada.

### **About VY-AADC**

VY-AADC, an investigational gene therapy, is designed to put the aromatic L-amino acid decarboxylase (AADC) enzyme into brain cells where it can convert levodopa to dopamine. To do this, the AADC gene is delivered inside a transporter called "adenoviral-associated viral vector" (AAV). Interim results from an open-label Phase 1b 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 and uterine fibroids\*. 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](http://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, including INGREZZA; the value INGREZZA and/or our product candidates may bring to patients; the continued success of the launch of INGREZZA; the collaboration with Voyager Therapeutics; and the timing of completion of our clinical, regulatory, and other development activities and those of our collaboration partners. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are: 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 third parties for development and manufacturing activities related to INGREZZA and the Company's product candidates, 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, opicapone, or the Company's other product candidates; 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 the benefits of the agreements with our collaboration partners may never be realized, including Voyager and BIAL; 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 March 31, 2019. Neurocrine disclaims any obligation to update the statements contained in this presentation after the date hereof.

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