# Neurocrine Biosciences to Present New Data from its Movement Disorder Programs for Tardive Dyskinesia and Parkinson's Disease at Upcoming Scientific Meetings

September 3, 2020

**-- New Three-Year Gene Therapy Data for Parkinson's Disease Part of 30 Abstracts to be Presented at 2020 Psych Congress and the MDS Virtual Congress 2020 --**

**- INGREZZA® (valbenazine) Data Analyses from Long-Term Phase III Studies Demonstrate Sustained Clinical Benefit, Safety and Tolerability in Patients with Tardive Dyskinesia**

**- New Phase III Data Analyses of ONGENTYS® (opicapone) Support Treatment as Add-On Therapy to Levodopa/Carbidopa for Parkinson's Patients with Motor Fluctuations**

**- New Phase Ib Data of Investigational Gene Therapy Compound, NBIb-1817 (VY-AADC), Evaluate Three-Year Safety and Clinical Outcomes of Patients with Advanced Parkinson's Disease**

SAN DIEGO, Sept. 3, 2020 /PRNewswire/ -- Neurocrine Biosciences, Inc. (Nasdaq: NBIX) today announced it will present new data as part of 30 abstracts accepted from its movement disorder programs for tardive dyskinesia and Parkinson's disease at the upcoming virtual scientific meetings, 2020 Psych Congress on September 10–13 and the MDS Virtual Congress 2020 on September 12–16.

Key highlights include data analyses from long-term Phase III studies of INGREZZA® (valbenazine) capsules in adult patients with tardive dyskinesia demonstrating sustained clinical benefit, safety and tolerability; new Phase III data analyses of ONGENTYS® (opicapone) capsules as an add-on therapy to levodopa/carbidopa in patients with Parkinson's disease experiencing motor "off" episodes, and new Phase Ib data of an investigational gene therapy, NBIb-1817 (VY-AADC), evaluating the three-year safety and clinical outcomes of patients with advanced Parkinson's disease.

"We are excited to present new data at this year's Psych Congress and MDS that illustrate the depth and breadth of our movement disorder programs for tardive dyskinesia and Parkinson's disease in clinical research," said Eiry W. Roberts, M.D., Chief Medical Officer at Neurocrine Biosciences. "These data for INGREZZA, ONGENTYS and our investigational gene therapy NBIb-1817 underscore our ongoing commitment to improve the lives of patients living with these movement disorders."

**2020 Psych Congress**

**INGREZZA® (valbenazine) Capsules**

- Onset and Resolution of Key Adverse Events in Valbenazine-Treated Patients with Tardive Dyskinesia: Pooled Post-Hoc Analyses from Two Long-Term Clinical Trials (Poster # 179)
- Item 8 of the Abnormal Involuntary Movement Scale (AIMS) as an Overall Index of Improvement in Patients with Tardive Dyskinesia (Poster # 166)
- Clinician-Reported Patient Awareness of Symptoms and Severity of Tardive Dyskinesia in Patients Prescribed VMAT2 Inhibitors (Poster # 122)
- Concerns from Community Mental Health Center (CMHC) Patients Regarding Drug-Induced Movement Disorders: Impact on Functioning and Treatment Beliefs (Poster # 126)
- Understanding the Evolving Continuing Medical Education Needs of Physicians Managing Patients with TD (Poster # 212)
- Knowledge of the Recognition and Management of Tardive Dyskinesia Markedly Improved Among Psychiatrists: Assessing the Impact of Online Medical Education (Poster # 167)
- TD360: Outcomes of a Tardive Dyskinesia Educational Curriculum – A Focus on Quality of Life, Increased Detection and Novel Treatments (Poster # 159)

**MDS Virtual Congress 2020**

**INGREZZA® (valbenazine) Capsules – Tardive Dyskinesia**

- Real-World Evaluation of Patient Characteristics and Disease Management in Long-Term Valbenazine Treatment in Adults with Tardive Dyskinesia (Poster # 99)
- Real-World Use and Impact of VMAT2 Inhibitors in Patients with Tardive Dyskinesia (Poster # 102)
- Patient Perspective of Tardive Dyskinesia: Results from a Social Media Listening Study (Poster # 101)

**ONGENTYS® (opicapone) Capsules – Parkinson's Disease (in collaboration with BIAL)**

- Opicapone as First-Line Adjunctive Levodopa Treatment in Parkinson's Disease Patients with Motor Fluctuations: Findings from BIPARK-I and BIPARK-II Combined Post-Hoc Analysis (Poster # 999)
- Opicapone's Added Benefit as a First-Line Adjunctive Therapy to Levodopa and When Used Promptly in the Motor Fluctuations Spectrum of Parkinson's Disease: a Post-Hoc Analysis of BIPARK-I and BIPARK-II (Poster # 994)
- Efficacy and Safety of Opicapone in Parkinson's Disease Patients According to Duration of Motor Fluctuations: Post-Hoc Analysis of BIPARK-I and BIPARK-II (Poster # 993)
- Super-Responders to Opicapone Adjunct Treatment to Levodopa in Parkinson's Disease Patients with Motor Fluctuations: Combined Post-Hoc Analysis of BIPARK-I and BIPARK-II (Poster # 974)
- Efficacy of Opicapone in Different Levodopa-Containing Treatment Regimens in Parkinson's Disease Patients with Motor Fluctuations (Poster # 973)
- Opicapone in Clinical Practice in Parkinson's Disease Patients with Motor Fluctuations: Findings from the OPTIPARK Study (Poster # 1049)
- Onset of Drug-Related Adverse Events in Parkinson's Disease Patients with Motor Fluctuations Treated with Opicapone in Clinical Practice: OPTIPARK Post-Hoc Analysis (Poster # 1029)
- Effect of Opicapone and Entacapone on Daily Pattern of Motor Fluctuations in Parkinson's Disease Patients (Poster # 1027)
- Effect of Opicapone and Entacapone on Levodopa Short Duration Response (Poster # 1030)
- Effect of Opicapone and Entacapone on Early Morning-OFF Pattern in Parkinson's Disease Patients with Motor Fluctuations (Poster # 1071)
- Efficacy and Safety/Tolerability of Opicapone in Catechol-O-Methyltransferase Inhibitor-Naïve Parkinson's Disease Patients Recently Diagnosed with Motor Fluctuations (Poster # 1028)
- Efficacy of Opicapone Compared to Entacapone in Catechol-O-Methyltransferase Inhibitor-Naïve Parkinson's Disease Patients Recently Diagnosed with Motor Fluctuations: a Post-Hoc Conservative Analysis (Poster # 998)
- Motor Fluctuations in a Real-World Sample of Patients with Parkinson's Disease (Poster # 343)
- Real-World Healthcare Resource Utilization in Patients with Parkinson's Disease and Motor Fluctuations (Poster # 344)
- Patient Characteristics, Treatment Patterns and Disease Burden in People with Parkinson's Disease: Insights from the Parkinson's Disease Real-World Impact Assessment (PRISM) Study (Poster # 1128)
- Impulse Control Behaviours in People with Parkinson's Disease: Findings from the Parkinson's Disease Real-World Impact Assessment (PRISM) Study (Poster # 695)
- Burden of Care-Partners of People with Parkinson's Disease: Findings from Parkinson's Disease Real-World Impact Assessment (PRISM) Study (Poster # 1143)
- Characterization of the Pattern of Daily Motor Fluctuations in Parkinson's Disease Patients Based on Home Diaries (Poster # 1011)

**Investigational NBIb-1817- Parkinson's Disease (in collaboration with Voyager)**

- Three-Year Safety and Clinical Outcomes from the PD-1101 Trial of AADC Gene Therapy for Advanced Parkinson's Disease – Poster # 879: Update on Genetics of Movement Disorders, September 13, 2020, 10:30–12:30pm EST (10-minute prerecorded presentation)
- AADC Gene Therapy Administered via a Posterior Approach: 24-month Results from the PD-1102 Trial in Advanced Parkinson's Disease – Poster # 889: Poster Tour, launches on-demand on September 11, 2020 8:00am EST (5-minute prerecorded presentation)

**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 Biosciences'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 Information**
**Approved Use**
INGREZZA® (valbenazine) capsules is a prescription medicine used to treat adults with movements in the face, tongue, or other body parts that cannot be controlled (tardive dyskinesia).

It is not known if INGREZZA is safe and effective in children.

**Important Safety Information**
**Do not take INGREZZA if you** are allergic to valbenazine, or any of the ingredients in INGREZZA.

**INGREZZA may cause serious side effects, including**:

- **Sleepiness (somnolence).** Do not drive, operate heavy machinery, or do other dangerous activities until you know how INGREZZA affects you.
- **Heart rhythm problems (QT prolongation).** INGREZZA may cause a heart problem known as QT prolongation.
- **Symptoms of QT prolongation may include**: fast, slow, or irregular heartbeat, shortness of breath, dizziness or fainting.
- **Parkinson-like symptoms**. Symptoms include: shaking, body stiffness, trouble moving or walking, or keeping your balance.

Tell your healthcare provider right away if you have a change in your heartbeat (a fast or irregular heartbeat), or if you faint.

**Before taking INGREZZA, tell your healthcare provider about all of your medical conditions including if you:** have liver or heart problems, are pregnant or plan to become pregnant, or are breastfeeding or plan to breastfeed.

**Tell your healthcare provider about all the medicines you take,** including prescription and over-the-counter medicines, vitamins and herbal supplements.

**The most common side effect of INGREZZA is sleepiness (somnolence). Other side effects include** changes in balance (balance problems, dizziness) or an increased risk of falls, headache, feelings of restlessness, dry mouth, constipation, and blurred vision.

These are not all of the possible side effects of INGREZZA. Call your doctor for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Please see INGREZZA full **Product Information**.

**About Parkinson's Disease**
Parkinson's disease is a chronic, progressive and debilitating neurodegenerative disorder that affects approximately one million people in the United States and six million people worldwide. Parkinson's disease is caused by low dopamine levels produced in the brain. Dopamine helps transmit signals between the areas of the brain that control all purposeful movements, including talking, walking and writing. As Parkinson's disease progresses, dopamine production steadily decreases, resulting in increased problems with motor symptoms including slowed movement (bradykinesia), tremor, rigidity, impaired posture and balance, and difficulty with speech and writing.

There is presently no cure for Parkinson's disease and management of the disease consists of the use of treatments that attempt to control motor symptoms primarily through dopaminergic mechanisms. The current gold standard for treatment of motor symptoms is levodopa/carbidopa. While levodopa/carbidopa improves patients' motor symptoms, as the disease progresses, the beneficial effects of levodopa begin to wear off more quickly. Patients then experience motor fluctuations throughout the day between "on" time, periods when the medication is working and Parkinson's disease symptoms are controlled, and "off" time, when the medication is not working and motor symptoms return.

**About ONGENTYS® (opicapone) Capsules**
ONGENTYS is a novel, once-daily, oral, peripheral, selective and reversible catechol-O-methyltransferase (COMT) inhibitor approved by the U.S. Food and Drug Administration (FDA) as an add-on treatment to levodopa/carbidopa in patients with Parkinson's disease experiencing "off" episodes. ONGENTYS inhibits the COMT enzyme, which breaks down levodopa, making more levodopa available to reach the brain.

In June 2016, BIAL – Portela & CA, S.A. (BIAL) received approval from the European Commission for ONGENTYS as an adjunct therapy to preparations of levodopa/DOPA decarboxylase inhibitors in adult patients with Parkinson's disease and end-of-dose motor fluctuations who cannot be stabilized on those combinations. BIAL currently markets ONGENTYS in Germany, United Kingdom, Spain, Portugal and Italy. Neurocrine Biosciences in-licensed opicapone from BIAL in 2017 and has exclusive

development and commercialization rights in the U.S. and Canada.

**Important Information**

**Approved Use**
ONGENTYS® (opicapone) capsules is a prescription medicine used with levodopa and carbidopa in people with Parkinson's disease (PD) who are having "OFF" episodes.
It is not known if ONGENTYS is safe and effective in children.

**Important Safety Information**
**Do not take ONGENTYS if you:**

- take a type of medicine called a non-selective monoamine-oxidase (MAO) inhibitor.
- have a tumor that secretes hormones known as catecholamines.

**Before taking ONGENTYS, tell your healthcare provider about all of your medical conditions, including if you**:

- have daytime sleepiness from a sleep disorder, have unexpected periods of sleep or sleepiness, or take a medicine to help you sleep or that makes you feel sleepy.
- have had intense urges or unusual behaviors, including gambling, increased sex drive, binge eating, or compulsive shopping.
- have a history of uncontrolled sudden movements (dyskinesia).
- have had hallucinations or psychosis.
- have liver or kidney problems.
- are pregnant or plan to become pregnant, or are breastfeeding or plan to breastfeed.

**Tell your healthcare provider about all the medicines you take**, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Especially tell your healthcare provider if you take nonselective MAO inhibitors (such as phenelzine, tranylcypromine, and isocarboxazid) or catecholamine medicines (such as isoproterenol, epinephrine, norepinephrine, dopamine, and dobutamine), regardless of how you take the medicine (by mouth, inhaled, or by injection).

ONGENTYS and other medicines may affect each other causing side effects. ONGENTYS may affect the way other medicines work, and other medicines may affect how ONGENTYS works.

**What should I avoid while taking ONGENTYS?**

- Do not drive, operate machinery, or do other dangerous activities until you know how ONGENTYS affects you.

**What are the possible side effects of ONGENTYS?**
**ONGENTYS may cause serious side effects, including:**

- **Falling asleep during normal activities** such as driving a car, talking or eating while taking ONGENTYS or other medicines used to treat Parkinson's disease, without being drowsy or without warning. This may result in having accidents. Your chances of falling asleep while taking ONGENTYS are higher if you take other medicines that cause drowsiness.
- **Low blood pressure or dizziness**, light headedness, or fainting.
- **Uncontrolled sudden movements (dyskinesia).** ONGENTYS may cause uncontrolled sudden movements or make such movements worse or happen more often.
- **Seeing, hearing, or feeling things that are not real (hallucinations), believing things that are not real (delusions)**, or aggressive behavior.
- **Unusual urges (impulse control and compulsive disorders)** such as urges to gamble, increased sexual urges, strong urges to spend money, binge eating, and the inability to control these urges.

Tell your healthcare provider if you experience any of these side effects or notice changes in your behavior.

**The most common side effects of ONGENTYS include** uncontrolled sudden movements (dyskinesia), constipation, increase in an enzyme called blood creatine kinase, low blood pressure, and weight loss.

These are not all of the possible side effects of ONGENTYS. Call your healthcare provider for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

**Please see ONGENTYS full Product Information.**

**About NBIb-1817 (VY-AADC)**
Dopamine is a chemical "messenger" that is produced in the brain and is involved in the control of movement. It is made in the brain when the enzyme AADC (Aromatic l-amino acid decarboxylase) converts the chemical levodopa to dopamine. As Parkinson's disease progresses, there is less AADC enzyme in parts of the brain where levodopa is converted to dopamine. This may be associated with patients' motor function worsening with less predictable response to medications.

NBIb-1817 is an investigational recombinant adeno-associated viral (AAV) vector serotype 2 encoding the gene for human AADC

that is designed to produce the AADC enzyme in brain cells where it can convert levodopa to dopamine. NBIb-1817 is infused into the putamen using intraoperative monitoring with magnetic resonance imaging (MRI)-facilitated targeted delivery.

In January 2019, Neurocrine Biosciences and Voyager Therapeutics announced a strategic collaboration focused on the development and commercialization of this and other gene therapy programs.

**About Neurocrine Biosciences**
Neurocrine Biosciences is a neuroscience-focused, biopharmaceutical company with 28 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, Parkinson's disease, endometriosis* and uterine fibroids*, with three pivotal and five mid-stage clinical programs in multiple therapeutic areas. 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: our future financial and operating performance; risks associated with the commercialization of INGREZZA and our other products; risks that the launch of ONGENTYS may be delayed; the impact of the COVID-19 pandemic and efforts to mitigate its spread on our business; risks and uncertainties associated with the scale and duration of the COVID-19 pandemic and resulting global, national, and local economic and financial disruptions; risk and uncertainties related to any COVID-19 quarantines, shelter-in-place and similar government orders that are currently in place or that may be put in place in the future, including the impact of such orders on our business operations and the business operations of the third parties on which we rely; risks related to the development of our product candidates; risks associated with our dependence on third parties for development and manufacturing activities related to INGREZZA and our product candidates, and our ability to manage these third parties; risks that the FDA or other regulatory authorities may make adverse decisions regarding our products or product candidates; risks associated with our dependence on BIAL for manufacturing activities for ONGENTYS, and our ability to manage BIAL; risks that clinical development activities may not be completed on time or at all, or may be delayed for regulatory, manufacturing, COVID-19 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 potential benefits of the agreements with our collaboration partners may never be realized; risks that our products, 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 our periodic reports filed with the SEC, including without limitation our quarterly report on Form 10-Q for the quarter ended June 30, 2020. Neurocrine disclaims any obligation to update the statements contained in this press release after the date hereof.

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