Neurocrine Biosciences Announces FDA Acceptance of New Drug Application for Opicapone as an Adjunctive Treatment for Patients with Parkinson's Disease

July 10, 2019

- FDA Accepts New Drug Application for Opicapone, a Once-Daily, Oral, Catechol-O-Methyltransferase (COMT) Inhibitor, as Adjunctive Therapy to Levodopa/Carbidopa

- Prescription Drug User Fee Act (PDUFA) Target Action Date Set for April 26, 2020

- New Drug Application Based on Data from 38 Clinical Studies, Including Two Phase III Studies, With More Than 1,000 Parkinson's Disease Patients Treated with Opicapone

SAN DIEGO, July 10, 2019 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) today announced that the U.S. Food and Drug Administration (FDA) has accepted its New Drug Application (NDA) for opicapone, a novel, once-daily, oral, selective catechol-O-methyltransferase (COMT) inhibitor as an adjunctive treatment to levodopa/carbidopa in patients with Parkinson's disease experiencing OFF episodes. Parkinson's disease is a chronic, progressive and debilitating neurodegenerative disorder that affects approximately one million people in the U.S. The FDA has set a standard 12-month review process with a Prescription Drug User Fee Act (PDUFA) target action date of April 26, 2020.

"People living with Parkinson's disease often struggle to control their motor fluctuations due to the progressive neurodegenerative effects of the disorder. With opicapone, we aim to prolong the benefits of levodopa by providing a new treatment option to patients with Parkinson's disease in the U.S.," said Eiry W. Roberts, M.D., Chief Medical Officer, Neurocrine Biosciences. "It is our goal to help patients maintain good ON time – the period when their motor symptoms are better controlled – and reduce OFF time – the period when the effects of levodopa have worn off. We look forward to working with the FDA to bring this new treatment option to patients coping with this debilitating disorder."

The NDA for opicapone is supported by data from 38 clinical studies, including two Phase III studies (BIPARK-1 and BIPARK-2), with more than 1,000 Parkinson's disease patients treated with opicapone. BIPARK-1 was a Phase III, randomized, double-blind placebo- and active-controlled study of opicapone as an adjunct to levodopa therapy in which approximately 600 patients with Parkinson's disease and motor fluctuations received once-daily opicapone (5 mg, 25 mg, or 50 mg), placebo, or 200 mg doses of the COMT inhibitor entacapone for 14 to 15 weeks. BIPARK-2 was a Phase III, randomized, double-blind placebo-controlled study of opicapone as an adjunct to levodopa therapy in which approximately 400 patients with Parkinson's disease and motor fluctuations received once-daily opicapone (25 mg or 50 mg) or placebo for 14 to 15 weeks. The primary endpoint in both studies was the change from baseline in absolute time in the OFF state, as assessed by patient diaries.

In June 2016, BIAL – Portela & CA, S.A. (BIAL) received approval from the European Commission for 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. 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. As a result of the FDA's acceptance of the NDA submission, Neurocrine Biosciences will pay a $10 million milestone payment to BIAL.

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 the disease 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 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, 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. Among the factors that could cause actual results to differ materially from those indicated in the forward-looking statements are: the risks and uncertainties the Company faces include risks that the opicapone NDA may not obtain regulatory approval from the FDA or such approval may be delayed or conditioned; risks that additional regulatory submissions may not occur or be submitted in a timely manner; risks or uncertainties related to the development of opicapone; risks and uncertainties relating to competitive products and technological changes that may limit demand for opicapone; 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 the FDA or other regulatory authorities may make adverse decisions regarding opicapone; 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 agreement with BIAL may never be realized; risks that 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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