Neurocrine Biosciences Reports First Quarter 2019 Financial Results

April 29, 2019

INGREZZA® (valbenazine) First Quarter Net Product Sales of $136.4 Million with Approximately 24,200 TRx
Opicapone New Drug Application Submission Remains on Track for Q2 2019
Neurocrine Biosciences Plans to Meet with FDA to Discuss Registration Program for Congenital Adrenal Hyperplasia in Q3 2019
Neurocrine Biosciences and Voyager Therapeutics Begin Collaboration on Strategic Gene Therapy Programs for Parkinson's Disease and Friedreich's Ataxia

SAN DIEGO, April 29, 2019 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) today announced its financial results for the quarter ended March 31, 2019 and provided an update on the launch of INGREZZA® (valbenazine) and its clinical development programs.

"We remain focused on building awareness of tardive dyskinesia and are encouraged by the continued adoption of INGREZZA among healthcare providers and the patient community. Importantly, a record number of new patients started treatment with INGREZZA during the first quarter," said Kevin Gorman, Ph.D., Chief Executive Officer of Neurocrine Biosciences. "Within our product pipeline, we remain on track for the NDA submission of opicapone for Parkinson's disease, plan to discuss the registration program for congenital adrenal hyperplasia with the FDA, and look forward to making progress on the Parkinson's disease and Friedreich's ataxia gene therapy programs in collaboration with Voyager Therapeutics. We have laid the foundation to make meaningful strides throughout 2019 and work towards our goal of having three FDA-approved treatments in four indications by 2020."

Financial Results

Total revenue was $138.4 million for the first quarter of 2019, compared to $71.1 million for the same period in 2018.

Total revenues were comprised of the following (*unaudited, in millions*):

	Three Months Ended March 31,	
	2019	**2018**
Revenues:		
INGREZZA product sales, net	$ 136.4	$ 71.1
Collaboration revenue	2.0	-
Total revenue	$ 138.4	$ 71.1

INGREZZA received U.S. Food and Drug Administration (FDA) approval in 2017, becoming the first medicine approved in the United States for the treatment of adults with tardive dyskinesia. ORILISSA® (elagolix) was approved by the FDA for the treatment of endometriosis with associated moderate to severe pain during the third quarter of 2018, with AbbVie sales beginning in August 2018.

The Company reported a net loss of $102.1 million, or $1.12 net loss per share, for the first quarter of 2019, compared to $41.8 million, or $0.47 net loss per share, for the same period in 2018. The increase in net loss for the first quarter of 2019 is primarily due to $113.1 million of in-process research and development (IPR&D) in connection with the strategic collaboration with Voyager. This was partially offset by increased INGREZZA net product sales.

Research and development (R&D) expenses decreased to $37.7 million for the first quarter of 2019, from $48.9 million for the same period in 2018. The decrease in R&D expenses is primarily due to a prior year $8 million non-recurring share-based compensation charge and a prior year $10 million event-based payment to BIAL – Portela & CA, S.A. (BIAL) for opicapone.

In connection with the closing of the Voyager transaction in March 2019, the Company made an upfront payment of $115 million

and purchased $50 million of Voyager's common stock. The Company accounted for this transaction, including related transaction costs, as an asset acquisition and expensed $113.1 million as IPR&D and recorded the $50 million equity investment at $54.7 million as an asset on the Company's balance sheet based upon the fair value at the timing of closing. The equity investment will be marked to market each quarter with gains and losses recorded to other income/expense.

Sales, general and administrative (SG&A) expenses increased to $87.5 million for the first quarter of 2019, from $58.6 million for the same period in 2018. The increase in SG&A expenses is primarily due to the sales force expansion in the third quarter of 2018, the national launch of a patient-focused disease state awareness campaign, Talk About TD, and an increase in the Branded Pharmaceutical Drug fee expense.

The Company's balance sheet at March 31, 2019, reflected total assets of $957.7 million, including cash and available for sale investments of $700.8 million, compared to total assets of $993.2 million at December 31, 2018.

Pipeline Highlights

ORILISSA® (elagolix) Update

On July 24, 2018, AbbVie, in collaboration with Neurocrine, announced FDA approval and in October 2018 Health Canada approval for ORILISSA for the management of endometriosis with associated moderate to severe pain. The FDA granted priority review to ORILISSA. The FDA grants priority review designation to medicines that, if approved, would provide a significant improvement in the safety or effectiveness of treatment of a serious condition. AbbVie began commercialization of ORILISSA in the United States in August 2018.

AbbVie provided positive top-line efficacy data from two Phase III studies in women with uterine fibroids in the first quarter of 2018 and from the associated six-month safety extension study during the third quarter of 2018. The ELARIS UF-I and UF-II studies of elagolix met all primary and ranked secondary endpoints at month six. These replicate Phase III studies were randomized, parallel, double-blind, placebo-controlled clinical trials evaluating elagolix alone or in combination with low-dose hormone (add-back) therapy in women with heavy uterine bleeding associated with uterine fibroids. The studies enrolled approximately 400 patients each for an initial six-month placebo-controlled dosing period. At the end of the six months of placebo-controlled evaluation, patients were eligible to enter an additional six-month safety extension study. The primary efficacy endpoint of the study was an assessment of the change in menstrual blood loss utilizing the alkaline hematin method comparing baseline to month six. Additional secondary efficacy endpoints were evaluated including the change in fibroid volume and hemoglobin. Bone mineral density was assessed via dual-energy x-ray absorptiometry (DEXA) scan at baseline at the conclusion of dosing and at six months post-dosing. Results from these studies will form the basis for an anticipated New Drug Application (NDA) submission to the FDA for the approval of elagolix in the treatment of uterine fibroids in the middle of 2019.

Opicapone Update

In February 2017, the Company entered into an exclusive licensing agreement with BIAL for the development and commercialization of opicapone in the United States and Canada. Opicapone is a once-daily, oral, peripherally-acting, highly-selective catechol-O-methyltransferase inhibitor, being developed as an adjunct therapy to preparations of levodopa/DOPA decarboxylase inhibitors for adult patients with Parkinson's disease and motor fluctuations. The Company met with the FDA in January 2018 and based upon the BIPARK-I and BIPARK-II pivotal Phase III studies conducted by BIAL, the FDA did not require additional Phase III trials to form an NDA submission. The Company remains on track for an NDA submission in the second quarter of 2019.

CAH Program (NBI-74788) Update

In the second quarter of 2017, the Company successfully completed the Phase I investigational new drug (IND)-opening study of NBI-74788 in healthy volunteer participants. The study was a randomized, open-label, two-period crossover study to evaluate the pharmacokinetics, the effect of food on pharmacokinetics, and the safety of NBI-74788 in a total of 16 healthy adults.

The Company began a Phase II proof-of-concept study examining the pharmacokinetics, pharmacodynamics, and safety of NBI-74788 in adult males and females with classic 21-hydroxylase deficiency CAH in November of 2017. This study will evaluate the safety and tolerability of NBI-74788 in patients with CAH together with the relationship between exposure and specific steroid hormone levels in these patients. In March 2019, positive interim results from this ongoing study demonstrated a clinically meaningful reduction in key biomarkers associated with the management of CAH. NBI-74788 was shown to be well tolerated with no serious adverse events reported to date. The Company plans to meet with the FDA during the third quarter of 2019 to discuss the registration program for NBI-74788 in adult and pediatric patients with CAH.

Voyager Collaboration and VY-AADC Program

Neurocrine Biosciences formed a strategic collaboration with Voyager Therapeutics focused on the development and commercialization of Voyager's 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. This collaboration combines Neurocrine's expertise in neuroscience, drug development and commercialization with Voyager's innovative gene therapy programs targeting severe neurological diseases. The collaboration became effective in March 2019 following the expiration of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976.

Based on the results from the VY-AADC Phase I programs in Parkinson's disease, RESTORE-1, a Phase II, randomized, placebo-surgery controlled, double-blinded, multi-center, clinical trial was initiated to evaluate the safety and efficacy of VY-AADC in patients who have been diagnosed with Parkinson's disease for at least four years, are not responding adequately to oral medications, and have at least three hours of OFF-time during the day as measured by a validated self-reported patient diary.

Conference Call and Webcast Today at 4:30 PM Eastern Time

Neurocrine will hold a live conference call and webcast today at 4:30 p.m. Eastern Time (1:30 p.m. Pacific Time). Participants can access the live conference call by dialing 877-876-9173 (US) or 785-424-1667 (International) using the conference ID: NBIX. The webcast can also be accessed on Neurocrine's website under Investors at www.neurocrine.com. A replay of the webcast will be available on the website approximately one hour after the conclusion of the event and will be archived for approximately one month.

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 from 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 (≥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 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. 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 and our partnered product, ORILISSA; the value INGREZZA, ORILISSA, and/or our product candidates may bring to patients; the continued success of the launch of INGREZZA; AbbVie's launch of ORILISSA; the planned submission of the NDA for opicapone; 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: the Company's future financial and operating performance; risks and uncertainties associated with the commercialization of INGREZZA and ORILISSA, including the likelihood of continued revenue and prescription growth of INGREZZA and ORILISSA; 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, ORILISSA, 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, ORILISSA, opicapone, or the Company's other product candidates; risks associated with the Company's dependence on AbbVie for the commercialization of ORILISSA and the development of elagolix; 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, BIAL, and Mitsubishi Tanabe; risks that INGREZZA, ORILISSA, 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 press release after the date hereof.

NEUROCRINE BIOSCIENCES, INC.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS
(in thousands, except per share data)
(unaudited)

	Three Months Ended March 31,	
	2019	**2018**
Revenues:		
Product sales, net	$ 136,431	$ 71,086
Collaboration revenue	1,972	-
Total revenues	138,403	71,086
Operating expenses:		
Cost of sales	1,129	950
Research and development	37,652	48,947
Acquired in-process research and development	113,081	-
Sales, general and administrative	87,538	58,636
Total operating expenses	239,400	108,533
Loss from operations	(100,997)	(37,447)
Other (expense) income:		
Interest expense	(7,853)	(7,504)
Unrealized gain on investment in restricted equity securities	1,680	-
Investment income and other, net	4,576	3,133
Total other expense, net	(1,597)	(4,371)
Loss before benefit from income taxes	(102,594)	(41,818)
Benefit from income taxes	(479)	-
Net loss	$ (102,115)	$ (41,818)
Net loss per share, basic and diluted	$ (1.12)	$ (0.47)
Weighted average common shares outstanding, basic and diluted	91,056	89,526

NEUROCRINE BIOSCIENCES, INC.
CONDENSED CONSOLIDATED BALANCE SHEETS
(in thousands)
(unaudited)

	March 31, 2019	**December 31, 2018**
Cash, cash equivalents and short-term investments	$ 524,068	$ 650,913
Other current assets	109,124	86,864
Total current assets	633,192	737,777
Property and equipment, net	36,661	33,869
Long-term investments	176,689	216,028
Investment in restricted equity securities	56,400	-
Operating lease assets	49,304	-

Restricted cash	5,477	5,477
Total assets	$ 957,723	$ 993,151
Current liabilities	$ 72,003	$ 88,233
Noncurrent operating lease liabilities	67,147	-
Convertible senior notes	393,435	388,496
Other long-term liabilities	15,863	35,657
Stockholders' equity	409,275	480,765
Total liabilities and stockholders' equity	$ 957,723	$ 993,151

View original content to download multimedia:http://www.prnewswire.com/news-releases/neurocrine-biosciences-reports-first-quarter-2019-financial-results-300840067.html

SOURCE Neurocrine Biosciences, Inc.

Neurocrine Biosciences, Inc., Navjot Rai (Media & Investors), 858-617-7623, IR@neurocrine.com