Neurocrine Biosciences Reports Year-End 2016 Results and Provides Investor Update for 2017

February 14, 2017

INGREZZATM New Drug Application PDUFA Action Date of April 11, 2017
Acquired U.S. Rights to Opicapone for Parkinson's Disease
Elagolix New Drug Application Submission for Endometriosis Expected in Third Quarter of 2017
Two Phase III Studies of Elagolix in Uterine Fibroids Underway, Top-Line Data End of Year
Phase II Study of Valbenazine in Pediatric Tourette Syndrome Expected to Readout in Second Quarter 2017
Phase II Trial for Essential Tremor Drug Candidate NBI-640756 Planned for 2017

SAN DIEGO, Feb. 14, 2017 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ:NBIX) today announced its financial results for the quarter and full year 2016, and highlighted recent progress on its pipeline.

"2016 was a very successful year for Neurocrine highlighted by the filing of our INGREZZA New Drug Application for tardive dyskinesia which was granted Priority Review by the FDA with a PDUFA date of April 11, 2017," said Kevin Gorman, Ph.D., Chief Executive Officer of Neurocrine Biosciences. "2017 will truly be a pivotal year for Neurocrine; we have been preparing all aspects of the company for the anticipated commercial launch of INGREZZA in the United States. We also in-licensed the U.S. commercial rights to opicapone for Parkinson's disease, a novel COMT inhibitor recently approved in Europe. In addition, our partner AbbVie is planning to submit the NDA for elagolix in endometriosis during the third quarter of this year and is also anticipating top-line data from the pivotal studies of elagolix in uterine fibroids at year end. Our 2017 plans put us firmly on track to achieve our goal of building a multi-product, commercial-stage company with a robust and sustainable pipeline."

For the fourth quarter of 2016, the Company reported a net loss of $44.7 million, or $0.51 loss per share, compared to a net loss of $29.3 million, or $0.34 loss per share for the same period in 2015. For the year ended December 31, 2016, the Company reported a net loss of $141.1 million, or $1.63 loss per share, as compared to a net loss of $88.9 million, or $1.05 loss per share for 2015. The increase in net loss for the fourth quarter and full year results primarily from increased research and development expenses in connection with the Company's advancing clinical stage pipeline, INGREZZA^{TM} (valbenazine) pre-commercialization activities for tardive dyskinesia and higher share-based compensation expense as detailed below.

The Company's balance sheet at December 31, 2016 reflected total assets of $365.1 million, including cash, investments and receivables of $352.1 million compared with balances at December 31, 2015 of $474.8 million and $464.3 million, respectively.

Research and development expenses were $22.6 million during the fourth quarter of 2016, compared to $21.8 million for the same period in 2015. For the year ended December 31, 2016, research and development expenses were $94.3 million, compared to $81.5 million for all of 2015. Quarterly research and development expense increased by approximately $0.8 million from 2015 to 2016 primarily due to expenses related to the Company's New Drug Application (NDA) for INGREZZA in tardive dyskinesia. The $12.8 million increase in research and development expenses from 2015 to 2016 was primarily due to expenses related to the Company's compilation and filing of its NDA for INGREZZA in tardive dyskinesia as well as higher external clinical development expenses related to INGREZZA, which is being evaluated in both tardive dyskinesia and Tourette syndrome.

General and administrative expenses increased from $8.9 million for the fourth quarter of 2015 to $23.7 million for the fourth quarter of 2016. For the year ended December 31, 2016 general and administrative expenses were $68.1 million, compared to $32.5 million for the prior year. The overall increase in general and administrative expense is primarily due to pre-commercialization activities for INGREZZA. Personnel related costs increased by $5.7 million quarter over quarter, and by $13.5 million from 2015 to 2016 primarily due to the expansion of sales and marketing and medical affairs personnel. Additionally, professional costs related to market research and other pre-commercial activities increased by $6.7 million quarter over quarter and by $15.1 million for 2016 compared to 2015.

2017 Financial Guidance

Revenues from milestones under the AbbVie agreement for 2017 are expected to be $30 million. Ongoing operating expenses for 2017 should approximate $230 million to $240 million, exclusive of the $30 million up-front fee for in-licensing opicapone. The 2017 anticipated expenses include an estimated $40 million of share-based compensation expense. The increase in expected expenses is primarily related to anticipated commercialization activities for INGREZZA in tardive dyskinesia.

Pipeline Highlights and Upcoming Events in 2017

INGREZZA (valbenazine) Update

The NDA for INGREZZA for the treatment of tardive dyskinesia was submitted to the U.S. Food and Drug Administration (FDA) during the third quarter of 2016. The NDA was accepted for Priority Review by the FDA and received a Prescription Drug User Fee Act (PDUFA) target action date of April 11, 2017.

During the third quarter of 2016, the Company completed the Kinect 3 study, a Phase III trial for tardive dyskinesia patients with underlying schizophrenia, schizoaffective disorder, bipolar or major depressive disorder who underwent six weeks of placebo-controlled assessment. Subsequent to the initial six weeks of treatment, subjects were eligible to continue in the Kinect 3 study for up to 42 weeks of additional INGREZZA treatment. The Company had previously announced positive efficacy results from the six-week placebo-controlled portion of the Kinect 3 study during the fourth quarter of 2015 as well as long-term safety and efficacy results in the fourth quarter of 2016.

The Company is currently conducting a separate one-year open-label safety study of INGREZZA, the Kinect 4 study. This study is fully enrolled and expected to complete its one year of dosing in early 2017.

The Company is also supporting an INGREZZA roll-over study for those patients who complete the one year of dosing in either the Kinect 3 or Kinect 4 studies. This roll-over study is designed to permit open-label access to INGREZZA for up to an additional 72 weeks of treatment.

INGREZZA is also being investigated in Tourette syndrome for both adult and pediatric patients.

The T-Forward study of adult Tourette syndrome patients recently reported top-line data. This randomized, double-blind, placebo-controlled, multi-dose, parallel group study enrolled 124 adults with moderate to severe Tourette's. The subjects received once-daily dosing of INGREZZA or placebo during the eight-week treatment period to assess the safety, tolerability and efficacy of INGREZZA. The primary endpoint of T-Forward was a change from baseline of placebo vs. active scores utilizing the Yale Global Tic Severity Scale (YGTSS) at the end of Week 8. The primary endpoint of YGTSS was not met at Week 8 (p=0.18), however the study showed a significant improvement in overall symptoms of Tourette as evidenced by the Clinical Global Impression of Change (p=0.015). Adverse events (AEs) in this trial were dose dependent and consistent with earlier clinical studies of INGREZZA.

The T-Force GREEN study is a randomized, double-blind, placebo-controlled, multi-dose, parallel group study that has enrolled approximately 90 children and adolescents. Pediatric Tourette subjects receive once-daily dosing of INGREZZA or placebo during a six-week treatment period to assess the safety, tolerability and efficacy of INGREZZA. The primary endpoint of this study is the change from baseline of the YGTSS between placebo and active treatment groups at the end of Week 6. Top-line data from this study is expected in the second quarter of 2017.

Additionally, the Company is also conducting an open-label, fixed-dose study of INGREZZA in up to 180 subjects with Tourette syndrome. This study is designed to enroll up to 90 children and adolescents and up to 90 adults who have completed either of the two placebo-controlled Tourette clinical trials: T-Force GREEN or T-Forward. This Phase II study will assess the long-term safety and tolerability of INGREZZA in children and adults with Tourette's.

Data from the Tourette studies is planned to be utilized to design a Phase III pivotal program for INGREZZA in treating Tourette syndrome.

Elagolix Update

AbbVie has completed the treatment portion of both Phase III studies of elagolix in endometriosis. The first study (Violet PETAL) has completed its off-drug follow-up period. The second study (Solstice) will complete the off-drug follow-up period during the second quarter of 2017. The top-line results from both trials were consistent showing that after six months of treatment, both doses of elagolix (150 mg once-daily and 200 mg twice-daily) met the study's co-primary endpoints of reducing scores of non-menstrual pelvic pain and menstrual pain (or dysmenorrhea) associated with endometriosis at month three, as well as month six, as measured by the Daily Assessment of Endometriosis Pain Scale. Among the most common AEs in both studies were hot flush, headache and nausea. While most AEs were similar across treatment groups some, such as hot flush and bone mineral density loss, were dose-dependent.

AbbVie is targeting an NDA submission with the FDA for elagolix in endometriosis during the third quarter of 2017.

In October 2016, AbbVie presented multiple scientific abstracts at the 72nd American Society for Reproductive Medicine Scientific Congress & Expo in Salt Lake City. The posters and oral presentations highlighted positive primary and secondary efficacy endpoint data from the Phase III studies of elagolix in premenopausal women who suffer from endometriosis as well as research on the economic burden of endometriosis and endometriosis-related surgery in women in the United States:

- *Elagolix, An Oral Gonadotropin-Releasing Hormone (GnRH) Antagonist, For The Management Of Endometriosis-Associated Pain: Safety And Efficacy Results From Two Double-Blind, Randomized, Placebo-Controlled Studies*; *Taylor H, et al.*
- *Use Of Elagolix For The Management Of Endometriosis-Associated Pain: Secondary Efficacy Results From Two Randomized, Placebo-Controlled Studies; Surrey, et al.*
- *The Effect of Elagolix On Bone Mineral Density: Safety Results From Two Randomized, Placebo-Controlled Studies In Women With Endometriosis-Associated Pain*; *Archer, et al.*
- *Incremental Costs of Healthcare and Work Loss Attributed to Endometriosis in a Cohort of Commercially Insured Women*; *Soliman, et al.*
- *Incidence of Comorbidities Among Women with Endometriosis: A Retrospective Matched Cohort Study*; *Soliman, et al.*
- *The Effect Of Elagolix On The Endometrium: Safety Results From Two Randomized, Placebo-Controlled Studies In Women With Endometriosis-Associated Pain; Diamond, et al*.
- *The Impact of Elagolix on Quality of Life in Women with Endometriosis-Associated Pain: Results From Two Randomized, Placebo-Controlled Studies Using the Endometriosis Health Profile Questionnaire; Taylor H, et al*.
- *Direct and Indirect Costs Associated with Endometriosis-Related Surgery Among Employed Women in the US; Soliman, et al*.

AbbVie is also currently conducting two replicate Phase III randomized, parallel, double-blind, placebo-controlled clinical trials evaluating elagolix alone or in combination with add-back therapy in women with heavy uterine bleeding associated with uterine fibroids. The studies are expected to enroll approximately 400 subjects each for an initial six-month placebo-controlled dosing period. At the end of the six-months of placebo-controlled evaluation, subjects are eligible to enter an additional six-month safety extension study. The primary efficacy endpoint of the study is an assessment of the change in menstrual blood loss utilizing the alkaline hematin method comparing baseline to month six. Additional secondary efficacy endpoints will be evaluated including assessing the change in fibroid volume and hemoglobin. Bone mineral density will be assessed via DXA scan at baseline, the conclusion of dosing and six months post-dosing. The Company expects the initial top-line efficacy data from the uterine fibroid Phase III program in late 2017. These two studies will form the basis for an anticipated 2019 submission with the FDA for the approval of elagolix in the treatment of uterine fibroids.

Opicapone Update

On February 9, 2017, we entered into an exclusive licensing agreement with BIAL – Portela & CA, S.A. (BIAL) for the development and commercialization of opicapone in the United States and Canada. Opicapone is a once-daily, peripherally-acting, highly-selective COMT inhibitor that was approved in June 2016 by the European Commission as an adjunct therapy to preparations of levodopa/DOPA decarboxylase inhibitors for adult patients with Parkinson's disease and end-of-dose motor fluctuations who cannot be stabilized on those combinations. We intend to meet with the FDA in 2017 to discuss a potential New Drug Application submission.

Essential Tremor Program (NBI-640756) Update

The Company has successfully completed an initial Phase I single site, randomized, double-blind, placebo-controlled, sequential dose-escalation, pharmacokinetic study assessing the safety and tolerability of a single dose of NBI-640756 in up to 32 healthy volunteers.

Based on the results of this initial study, the Company initiated a second Phase I, single site, randomized, double-blind, placebo-controlled, multiple-dose, sequential dose-escalation study to evaluate the safety, tolerability and pharmacokinetics of NBI-640756 in up to 30 healthy volunteers over a week of continuous dosing. The study is being conducted in multiple sequential cohorts of ten subjects per cohort; data from this second Phase I study is expected later in 2017. The data from this study, in conjunction with the single dose Phase I study and preclinical studies, will be evaluated and utilized in the design of the anticipated Phase II program for NBI-640756 in subjects with essential tremor.

Congenital Adrenal Hyperplasia Program (NBI-74788) Update

The Company has submitted an Investigational New Drug application with the FDA for its CRF receptor antagonist NBI-74788 to treat patients with classic congenital adrenal hyperplasia (CAH). The Company intends to initiate a Phase I safety and pharmacokinetics study exploring NBI-74788 in healthy volunteers during 2017.

Conference Call and Webcast Today at 5:00PM Eastern Time

Neurocrine will hold a live conference call and webcast today at 5:00 p.m. Eastern Time (2:00 p.m. Pacific Time). Participants can access the live conference call by dialing 888-632-3382 (US) or 785-424-1677 (International) using the conference ID NBIX. The call can also be accessed via the webcast through the Company's website at http://www.neurocrine.com.

If you are unable to attend the webcast and would like further information on this announcement please contact the Investor Relations Department at Neurocrine Biosciences at (858) 617-7600. A replay of the conference call will be available approximately one hour after the conclusion of the call by dialing 800-839-6136 (US) or 402-220-2572 (International) using the conference ID NBIX. The call will be archived for one month.

Neurocrine Biosciences, Inc. discovers and develops innovative and life-changing pharmaceuticals, in diseases with high unmet medical needs, through its novel R&D platform, focused on neurological and endocrine based diseases and disorders. The Company's two lead late-stage clinical programs are elagolix, a gonadotropin-releasing hormone antagonist for women's health that is partnered with AbbVie Inc., and INGREZZA, a vesicular monoamine transporter 2 inhibitor for the treatment of movement disorders. Neurocrine plans to commercialize INGREZZA in the United States upon approval of the NDA by the FDA.

Neurocrine Biosciences, Inc. news releases are available through the Company's website via the internet at http://www.neurocrine.com.

In addition to historical facts, this press release may contain 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 risks and uncertainties associated with Neurocrine's business and finances in general. Specifically, the risks and uncertainties the Company faces include risks that INGREZZA or the Company's other product candidates may not obtain regulatory approval or that the U.S. Food and Drug Administration or regulatory authorities outside the U.S. may make adverse decisions regarding the Company's product candidates; risks associated with the Company's dependence on AbbVie for the development and commercialization 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 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 the Company's product candidates may be precluded from commercialization by the proprietary rights of third parties, or have unintended side effects, adverse reactions or incidents of misuse; risks associated with the Company's dependence on BIAL for tech transfer, development and manufacturing activities related to opicapone; risks associated with the Company's dependence on other third parties for development, manufacturing and marketing activities; risks that the Company will be unable to raise additional funding required to complete development of all of its product candidates; risk and uncertainties relating to competitive products and technological changes that may limit demand for the Company's products; and other risks described in the Company's annual report on Form 10-K for the year ended December 31, 2015 and quarterly reports on Form 10-Q for the quarters ended March 31, 2016, June 30, 2016 and September 30, 2016. 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 December 31,		Year Ended December 31,	
	2016	2015	2016	2015
Revenues:				
Milestones and license fees	$ -	$ -	$ 15,000	$ 19,769
Total revenues	-	-	15,000	19,769
Operating expenses:				
Research and development	22,583	21,809	94,291	81,491

General and administrative	23,668	8,939	68,081	32,480
Total operating expenses	46,251	30,748	162,372	113,971
Loss from operations	(46,251)	(30,748)	(147,372)	(94,202)
Other income:				
Gain on sale/disposal of assets	-	-	8	9
Deferred gain on real estate	863	838	3,423	3,325
Investment income, net	716	584	2,838	1,928
Other income, net	13	11	13	11
Total other income	1,592	1,433	6,282	5,273
Net loss	$ (44,659)	$ (29,315)	$ (141,090)	$ (88,929)
Net loss per common share:				
Basic and Diluted	$ (0.51)	$ (0.34)	$ (1.63)	$ (1.05)
Shares used in the calculation of net loss per common share:				
Basic and Diluted	86,874	86,184	86,713	84,496

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

	December 31, 2016	December 31, 2015
Cash, cash equivalents and short-term investments	$ 307,350	$ 379,191
Other current assets	3,092	4,883
Total current assets	310,442	384,074
Property and equipment, net	6,271	3,432
Long-term investments	43,490	82,488
Restricted cash	4,883	4,791
Total assets	$ 365,086	$ 474,785
Current liabilities	$ 30,414	$ 25,715
Long-term liabilities	19,795	24,616
Stockholders' equity	314,877	424,454
Total liabilities and stockholders' equity	$ 365,086	$ 474,785

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/neurocrine-biosciences-reports-year-end-2016-results-and-provides-investor-update-for-2017-300407312.html

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