# Neurocrine Biosciences Reports Third Quarter 2016 Results

November 2, 2016

**-INGREZZA™ NDA for Tardive Dyskinesia Accepted for Priority Review by U.S. FDA-**
**-Positive Elagolix Data from Endometriosis Studies Presented at American Society for Reproductive Medicine-**
**-Enrollment Completed for Adult Study of INGREZZA in Tourette Syndrome-**

SAN DIEGO, Nov. 2, 2016 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ:NBIX) today announced its financial results for the quarter ended September 30, 2016. For the third quarter of 2016, the Company reported a net loss of $36.9 million, or $0.43 loss per share, compared to a net loss of $34.4 million, or $0.40 loss per share, for the same period in 2015. For the nine months ended September 30, 2016, the Company reported a net loss of $96.4 million, or $1.11 loss per share, as compared to net loss of $59.6 million, or $0.71 loss per share, for the first nine months of last year.

The Company's balance sheet at September 30, 2016 reflected total assets of $399.2 million, including cash, cash equivalents, investments and receivables of $385.3 million.

"Our NDA for INGREZZA™ in tardive dyskinesia was recently accepted by the FDA for Priority Review and we look forward to continuing our work with the FDA to potentially bring this important treatment option to patients and physicians," said Kevin Gorman, President and Chief Executive Officer of Neurocrine Biosciences. "In addition, our partner AbbVie presented Phase III data for elagolix in endometriosis at the 72nd American Society for Reproductive Medicine Scientific Congress & Expo. The results from the Phase III Violet Petal and Solstice clinical trials demonstrate that elagolix has the potential to be an important treatment for women suffering from endometriosis."

Research and development expenses were $20.9 million during the third quarter of 2016 compared to $24.4 million for the same period in 2015. Quarterly external development expense decreased by approximately $2.5 million from 2015 to 2016 primarily due to the completion of certain INGREZZA (valbenazine) manufacturing activities and the conclusion of the Kinect 3 clinical trial in 2016. Additionally, share-based compensation expense for research and development was $3.6 million lower during the third quarter of 2016 compared to the third quarter of 2015. The decrease in share-based compensation expense is primarily due to certain performance-based restricted stock units which met the criteria for expensing during the third quarter of 2015.

For the nine months ended September 30, 2016, research and development expenses were $71.7 million, compared to $59.7 million for the same period last year. This increase was primarily due to expenses related to the Company's compilation and submission of the New Drug Application (NDA) for INGREZZA in tardive dyskinesia. Additionally, external clinical development expenses related to INGREZZA, which is being evaluated in both tardive dyskinesia and Tourette syndrome, accounted for $4.0 million of the increase in year-to-date expenses.

General and administrative expenses increased from $11.5 million for the third quarter of 2015 to $17.5 million for the third quarter of 2016. For the nine months ended September 30, 2016, general and administrative expenses were $44.4 million, compared to $23.5 million for the first nine months of 2015. The overall increase in general and administrative expense is primarily due to pre-commercialization activities for INGREZZA. General and administrative share-based compensation expense was $4.0 million lower during the third quarter of 2016 compared to the third quarter of 2015. The decrease in quarterly share-based compensation expense is primarily due to certain performance-based restricted stock units which met the criteria for expensing during the third quarter of 2015. Other personnel related costs increased by $3.8 million quarter over quarter, and by $7.6 million for the first nine months of 2015 compared to the first nine months of 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 $5.4 million quarter over quarter and by $11.9 million for the first nine months of 2016 compared to the same period in 2015.

**Updated 2016 Financial Guidance**

The Company expects to end 2016 with approximately $340 million in cash, investments and receivables. The previous financial guidance was to end 2016 with approximately $320 million in cash, investments and receivables. Total expenses for 2016 are expected to be approximately $160 to $170 million. The previous financial guidance for 2016 expenses ranged from $185 to $195 million. The lower than anticipated cost of preclinical and clinical development as well as lower headcount contributed to this reduction in estimated 2016 expenses.

***Pipeline Highlights***

**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 that included moderate to severe tardive dyskinesia in 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.

The Company is currently conducting a separate one-year open-label safety study of INGREZZA, Kinect 4. 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 through two ongoing placebo-controlled Phase II Tourette syndrome studies in adults and pediatrics, the T-Forward study and T-Force GREEN study, respectively.

The T-Forward study is a randomized, double-blind, placebo-controlled, multi-dose, parallel group study that has completed enrollment of 124 adults with moderate to severe Tourette's. The adult Tourette patients are receiving 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 this study is a change from baseline of placebo vs. active scores utilizing the Yale Global Tic Severity Scale at the end of Week 8. Top-line data from this study is expected in January 2017.

The T-Force GREEN study is a randomized, double-blind, placebo-controlled, multi-dose, parallel group study of up to 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 Yale Global Tic Severity Scale between placebo and active treatment groups at the end of Week 6. Top-line data from this study is expected in early 2017.

Additionally, the Company has also launched 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.

**Elagolix Update**

In October, 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.

During the first quarter of 2016, AbbVie announced positive top-line results from the second of two Phase III clinical trials, the Solstice Study, a multinational study designed to evaluate the efficacy and safety of elagolix in 815 premenopausal women with endometriosis. The top-line results from this trial were consistent with those of the initial Phase III clinical trial, the Violet Petal Study, where 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. The observed safety profile of elagolix in the Solstice Study was consistent with observations from prior studies. Among the most common adverse events (AEs) 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 a 2017 NDA filing with the FDA for elagolix in endometriosis.

In early 2016, AbbVie announced the initiation of the Phase III uterine fibroids program consisting of two replicate 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 2017.

**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 2016. 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.

**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 800-839-2389 (US) or 402-220-7204 (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, as well as risks and uncertainties associated with the Company's R & D pipeline and the Company overall. Specifically, the risks and uncertainties the Company faces include risks that the Company's 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 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 third parties for development, manufacturing and marketing activities; risks that the Company's research programs will not identify pre-clinical candidates for further development; 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 and June 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 September 30, | | Nine Months Ended September 30, | |
|---|---|---|---|---|
| | **2016** | **2015** | **2016** | **2015** |
| Revenues: | | | | |
| License fees and milestones | $ - | $ - | $ 15,000 | $ 19,769 |
| Total revenues | - | - | 15,000 | 19,769 |
| Operating expenses: | | | | |
| Research and development | 20,942 | 24,388 | 71,708 | 59,682 |
| General and administrative | 17,494 | 11,456 | 44,413 | 23,541 |
| Total operating expenses | 38,436 | 35,844 | 116,121 | 83,223 |
| Loss from operations | (38,436) | (35,844) | (101,121) | (63,454) |
| Other income: | | | | |

| | | | | |
|---|---|---|---|---|
| (Loss) gain on sale/disposal of assets | (9) | - | 8 | 9 |
| Deferred gain on real estate | 853 | 828 | 2,560 | 2,487 |
| Investment income, net | 705 | 581 | 2,122 | 1,344 |
| Total other income | 1,549 | 1,409 | 4,690 | 3,840 |
| Net loss | $ (36,887) | $ (34,435) | $ (96,431) | $ (59,614) |
| Net loss per common share: | | | | |
| Basic and diluted | $ (0.43) | $ (0.40) | $ (1.11) | $ (0.71) |
| Shares used in the calculation of net loss per common share: | | | | |
| Basic and diluted | 86,784 | 85,856 | 86,659 | 83,927 |

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

| | September 30, 2016 | December 31, 2015 |
|---|---|---|
| Cash, cash equivalents and short-term marketable securities | $ 353,042 | $ 379,191 |
| Other current assets | 4,849 | 4,883 |
| Total current assets | 357,891 | 384,074 |
| Property and equipment, net | 6,180 | 3,432 |
| Long-term investments, available for sale | 30,207 | 82,488 |
| Restricted cash | 4,883 | 4,791 |
| Total assets | $ 399,161 | $ 474,785 |
| Current liabilities | $ 26,666 | $ 25,715 |
| Long-term liabilities | 20,844 | 24,616 |
| Stockholders' equity | 351,651 | 424,454 |
| Total liabilities and stockholders' equity | $ 399,161 | $ 474,785 |

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/neurocrine-biosciences-reports-third-quarter-2016-results-300356003.html

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