# Neurocrine Biosciences Reports First Quarter 2015 Results

April 30, 2015

**- Successful Secondary Offering-Company has over $500 Million Available to Fund Pipeline-**
**-Positive Top-Line Efficacy from First Elagolix Endometriosis Phase III Study-**
**-AbbVie Completes Recruiting for Elagolix Uterine Fibroids Phase IIB Study-**
**-NBI-98854 Partnered with Mitsubishi Tanabe in Asia for Movement Disorders-**

SAN DIEGO, April 30, 2015 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ:NBIX) today announced its financial results for the quarter ended March 31, 2015. For the first quarter of 2015, the Company reported a net loss of $1.2 million, or $0.01 loss per share, compared to a net loss of $11.8 million, or $0.17 loss per share, for the same period in 2014.

The Company's balance sheet at March 31, 2015 reflected cash, cash equivalents, investments and receivables of $517.9 million compared to $232.6 million at December 31, 2014. During the quarter, the Company completed a public offering of eight million shares of common stock that resulted in net proceeds of approximately $270 million. Additionally, the Company entered into a collaboration and license agreement with Mitsubishi Tanabe Pharma Corporation for development and commercialization of its VMAT2 inhibitor, NBI-98854, in Japan and other select Asian markets resulting in a $30 million up-front payment.

"The first quarter of 2015 was very successful for Neurocrine; starting with positive top-line elagolix Phase III data in endometriosis, a successful capital raise and signing an Asian partnership with Mitsubishi Tanabe for NBI-98854. This capital raise provides the funding necessary to continue to advance our pipeline, including moving NBI-98854 into commercialization upon successful completion of the Phase III program and FDA approval," said Kevin Gorman, Ph.D., President and Chief Executive Officer of Neurocrine Biosciences. "For the balance of 2015, we turn our focus to executing across our pipeline and advancing a novel compound from research into the clinic. Over the coming eight months, we look forward to seven clinical trials reading out across four distinct programs."

The $19.8 million of revenue for the first quarter of 2015 represents recognized revenue in the form of license fees from the NBI-98854 collaboration and license agreement with Mitsubishi Tanabe that was executed on March 31, 2015. Research and development expenses increased to $16.6 million during the first quarter of 2015 from $8.6 million during the same period in 2014. This increase was primarily due to higher external clinical development expenses and associated internal costs related to the Company's VMAT2 inhibitor, NBI-98854, which initiated Phase III development in the second half of 2014. Additionally, expenses related to the Company's congenital adrenal hyperplasia program increased from the first quarter of 2014. General and administrative expenses increased from $4.2 million in the first quarter of 2014 to $5.5 million for the first quarter of 2015, primarily due to higher personnel related costs, including a $0.5 million increase in share-based compensation expense.

***Pipeline Highlights***

**VMAT2 Update**

In 2014, the Company initiated a Phase III study of NBI-98854, the Kinect 3 study. The Kinect 3 study, along with the previous efficacy studies of NBI-98854, is designed to complete the placebo-controlled clinical efficacy evaluation of NBI-98854 in tardive dyskinesia. The primary endpoint in the Kinect 3 study is the mean change from baseline in the Abnormal Involuntary Movement Scale (AIMS) as assessed by blinded central raters. The Kinect 3 study includes approximately 240 subjects randomized to either placebo, once daily 40mg of NBI-98854, or once daily 80mg of NBI-98854 for six weeks of placebo-controlled dosing followed by an extension of active dosing through Week 48. Top-line efficacy data from the initial six weeks of placebo-controlled dosing is expected in the second half of 2015.

A separate one-year open-label safety study of NBI-98854 has also been initiated to support the anticipated 2016 filing of a New Drug Application in tardive dyskinesia.

As announced previously, Neurocrine has also received Breakthrough Therapy Designation from the FDA for NBI-98854 in the treatment of tardive dyskinesia.

The Company is also exploring NBI-98854 in an initial Tourette syndrome clinical trial, the T-Force study. This study is an open-label, multi-dose, two-week evaluation of 36 subjects with Tourette syndrome. Children and adolescents enrolled in the trial are receiving a once-daily dose of NBI-98854 during a two-week treatment period to assess both the safety and tolerability of NBI-98854. Additionally, the Yale Global Tic Severity Scale and the Premonitory Urge for Tics Scale are being utilized during the study to assess the impact of NBI-98854 on the patients' Tourette symptoms. Data read out from the T-Force study is expected in the second half of 2015.

On March 31, 2015 the Company entered into an exclusive collaboration and licensing agreement for the development and commercialization of its VMAT2 inhibitor, NBI-98854, in Japan and other select Asian markets with Mitsubishi Tanabe Pharma Corporation. Under the terms of the agreement, Neurocrine will receive an initial payment of $30 million and is eligible to receive up to $85 million in additional milestone payments associated with the development and commercialization of NBI-98854 in Asia. Upon commercialization, Neurocrine will receive royalties on product sales from select territories in Asia. Neurocrine will also support Mitsubishi Tanabe's clinical efforts in developing NBI-98854 for patients suffering from the chorea associated with Huntington's disease and tardive dyskinesia.

**Elagolix Update**

During the first quarter of 2015, AbbVie announced positive top-line results from the first of two ongoing Phase III clinical trials, the Violet Petal Study, designed to evaluate the efficacy and safety of elagolix in premenopausal women with endometriosis. Results from the trial show 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 ($p<0.001$) of reducing scores of non-menstrual pelvic pain (NMPP) 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 Violet Petal Study was consistent with observations from prior studies. Among the most common adverse events (AEs) were hot flush, headache, nausea and fatigue. While most AEs were similar across treatment groups some, such as hot flush and bone mineral density (BMD) loss, were dose-dependent.

AbbVie is also conducting the second Phase III study of elagolix for endometriosis, the Solstice Study. This study is similar in design to the Violet Petal Study and will assess 788 women, age 18 to 49, with moderate to severe endometriosis-associated pain at more than 200 sites globally. Top-line efficacy data from this study is expected in late 2015.

Elagolix is also being evaluated in women with uterine fibroids. AbbVie is conducting a Phase IIb clinical trial evaluating the change in menstrual blood loss of 520 women, age 18-51, with heavy menstrual bleeding associated with uterine fibroids. Patient recruitment has recently been completed and top-line data from this study is expected in 2015.

**Corticotropin Releasing Factor (Congenital Adrenal Hyperplasia) Update**

The Company recently announced the completion of a pilot clinical trial of NBI-77860 against placebo in adult females with refractory classic congenital adrenal hyperplasia (CAH). This eight person single dose exploratory study showed that NBI-77860 was effective in reducing the key biomarkers of adrenocorticotropic hormone (ACTH) and 17-hydroxyprogesterone androgen (17-OHP). A full description of the study results and related data was presented at the Endocrine Society's 97$^{th}$ Annual Meeting in San Diego on March 5, 2015.

Neurocrine has initiated a second clinical trial assessing three doses of NBI-77860 in an open-label, sequential cohort, single ascending dose pharmacokinetic/pharmacodynamic study. Fifteen adolescent females with classic CAH will be split into three cohorts and each will receive one dose of NBI-77860 once a day. Biomarker measurements include ACTH, 17-OHP, androgen and cortisol levels collected the morning after dosing. Data from this study is expected later in 2015.

**Conference Call and Webcast Today at 8:00AM Eastern Time**

Neurocrine will hold a live conference call and webcast today at 8:00 a.m. Eastern Time (5:00 a.m. Pacific Time). Participants can access the live conference call by dialing 866-952-1906 (US) or 785-424-1825 (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 800-839-5127(US) or 402-220-2692(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 NBI-98854, a vesicular monoamine transporter 2 inhibitor for the treatment of movement disorders. Neurocrine intends to maintain certain commercial rights to its VMAT2 inhibitor for evolution into a fully-integrated pharmaceutical company.

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 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 regulatory submissions may not occur or be submitted in a timely manner; 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 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 and sales activities; risks associated with the dependence on Mitsubishi Tanabe for development and commercialization of NBI-98854 in certain Asian countries; 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, 2014. 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)**

| | Three Months Ended March 31, | |
|---|---|---|
| | 2015 | 2014 |
| | (unaudited) | |
| Revenues: | | |
| License fees | $ 19,769 | $ - |
| Total revenues | 19,769 | - |
| Operating expenses: | | |
| Research and development | 16,575 | 8,572 |
| General and administrative | 5,482 | 4,153 |
| Total operating expenses | 22,057 | 12,725 |
| Loss from operations | (2,288) | (12,725) |
| Other income: | | |
| Interest and other income | 257 | 89 |
| Gain on sale of assets, net | 839 | 794 |
| Total other income | 1,096 | 883 |
| Net loss | $ (1,192) | $ (11,842) |
| Net loss per common share: | | |
| Basic and Diluted | $ (0.01) | $ (0.17) |
| Shares used in the calculation of net loss per common share: | | |
| Basic and Diluted | 80,349 | 70,260 |

**NEUROCRINE BIOSCIENCES, INC.**

**Condensed Consolidated Balance Sheets**
**(in thousands)**

| | March 31, 2015 | December 31, 2014 |
|---|---|---|
| | (unaudited) | |
| Cash, cash equivalents and short-term marketable securities | $370,007 | $ 193,809 |
| Other current assets | 34,946 | 4,394 |
| Total current assets | 404,953 | 198,203 |
| Property and equipment, net | 2,543 | 2,507 |
| Long-term investments | 115,452 | 37,492 |
| Restricted cash | 4,831 | 4,831 |
| Total assets | $527,779 | $243,033 |
| Current liabilities | $15,187 | $ 15,664 |
| Long-term liabilities | 27,862 | 18,670 |
| Stockholders' equity | 484,730 | 208,699 |
| Total liabilities and stockholders' equity | $527,779 | $243,033 |

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/neurocrine-biosciences-reports-first-quarter-2015-results-300074799.html

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