Neurocrine Biosciences Announces Expansion of its Clinical Pipeline

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Phase I/II Clinical Trial of CRF Antagonist in Adolescent Females with Classic Congenital Adrenal Hyperplasia
Initial Pilot Clinical Study Demonstrated Profound Impact on Key Biomarkers

SAN DIEGO, Dec. 10, 2014 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) announced today that NBI-77860, a proprietary corticotropin releasing factor 1 (CRF) receptor antagonist, has entered clinical trials for the treatment of classic congenital adrenal hyperplasia (CAH) a disease that affects approximately 20,000-30,000 people in the United States. The Company has successfully completed a pilot clinical trial in adults with classic CAH and is initiating an open-label, single ascending dose trial in approximately fifteen adolescent females with classic CAH, the 1401 Study.

"We are very pleased with the results from our pilot study of NBI-77860 in patients that was conducted earlier this year and excited to add another significant program to our clinical development pipeline," said Christopher F. O'Brien, Chief Medical Officer of Neurocrine Biosciences. "We have requested orphan drug status from the FDA while we continue to expand our preclinical and clinical efforts around NBI-77860 in classic CAH."

"I am very pleased with the data generated from this initial exploratory study; it looks very promising," said Dr. Richard Auchus, Professor of Internal Medicine, Division of Metabolism, Endocrinology & Diabetes at University of Michigan Health System. "This treatment strategy, if validated in future clinical trials, has the potential to revolutionize the treatment for CAH patients and make an important difference in their lives."

Results from the Initial Pilot Clinical Study

The pilot clinical trial was a blinded, single-site, pharmacokinetic/pharmacodynamic study assessing two single, ascending doses of NBI-77860 against placebo in adult females with refractory CAH. The eight study participants visited the investigative site for three separate overnight visits consisting of bedtime dosing with placebo or one of two active doses of NBI-77860. Each of the visits was separated by a three-week washout period. Key biomarker measurements included adrenocorticotropic hormone (ACTH), 17-hydroxyprogesterone (17-OHP), androgen, and cortisol levels collected in the morning after dosing. Data from this initial single dose exploratory study demonstrated a robust decrease in ACTH and 17-OHP.

A full description of the study results and related data will be presented at an upcoming scientific meeting.

1401 Study Design

The 1401 study is a Phase I/II open-label, sequential cohort, single ascending dose pharmacokinetic/pharmacodynamic study assessing three doses of NBI-77860. The fifteen adolescent females with classic CAH will be split into three cohorts and each will receive one dose of NBI-77860 at bedtime. Biomarker measurements include ACTH, 17-OHP, androgen, and cortisol levels collected the morning after dosing.

About Classic Congenital Adrenal Hyperplasia (CAH)

Classic CAH is a genetic disorder that results in an enzyme deficiency altering the production of adrenal steroids. Because of this deficiency, the adrenal glands have little to no cortisol biosynthesis resulting in a potentially life-threatening condition. If left untreated, classic CAH can result in salt wasting, dehydration and eventually death. Even with cortisol replacement, persistent elevation of ACTH from the pituitary gland results in excessive androgen levels leading to virilization of females including precocious puberty, menstrual irregularity, short stature, hirsutism, acne and fertility problems.

Corticosteroids are the current standard of care for classic CAH which are used to both correct the endogenous cortisol deficiency and reduce the excessive ACTH levels and androgen excess. However, the dose and duration of steroid use required to suppress ACTH is well above the normal physiological level of cortisol; resulting in metabolic syndrome, bone loss, growth impairment, and Cushing's syndrome as common and serious side effects.

Additional information on CAH can be obtained from the National Institutes of Health http://www.nlm.nih.gov, and patient advocacy groups such as CARES http://www.caresfoundation.org.

About NBI-77860

NBI-77860 is a potent, selective non-peptide CRF receptor antagonist as demonstrated in a range of in vitro/in vivo assays and human clinical studies. Blockade of CRF receptors at the pituitary has been shown to decrease the release of ACTH, which in turn

decreases the production of adrenal steroids including androgens, and potentially the symptoms associated with classic CAH. Lower ACTH levels would also reduce the amount of exogenous corticosteroid necessary for classic CAH patients to thrive avoiding the side-effects currently associated with excessive steroid therapy.

About Neurocrine Biosciences

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 a wholly owned 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 CRF program and Company overall. Specifically, the risks and uncertainties the Company faces with respect to the Company's CRF program include, but are not limited to; risk that NBI-77860 will not proceed to later stage clinical trials and risk that the Company's clinical trials will fail to demonstrate that NBI-77860 is safe and effective, risk that NBI-77860 may not replicate the results observed in our prior clinical development, risk that NBI-77860 may cause other unanticipated adverse effects, in the 1401 study or subsequent clinical trials, the risk of cessation or delay of the 1401 study or any ongoing or planned preclinical or clinical development activities for a variety of reasons, including additional information that may be requested or additional obligations that may be imposed by the FDA as a condition to our commencement and continuation of clinical trials with NBI-77860, and risk that NBI-77860 will not receive Orphan Drug designation. With respect to its pipeline overall, the Company faces risk that it will be unable to raise additional funding required to complete development of its product candidates; risk relating to the Company's dependence on contract manufacturers for clinical drug supply; risks associated with the Company's dependence on corporate partners for development, commercial manufacturing and marketing and sales activities for the Company's partnered programs; uncertainties relating to patent protection and intellectual property rights of third parties; risks and uncertainties relating to competitive products and technological changes that may limit demand for the Company's products; and overall risks associated with the process of discovering, developing and commercializing drug candidates that are safe and effective for use as human therapeutics and the other risks described in the Company's reports on Form 10-K for the year ended December 31, 2013 and on Form 10-Q for each of the quarters ended March 31, 2014, June 30, 2014 and September 30, 2014. Neurocrine undertakes no obligation to update the statements contained in this press release after the date hereof.

To view the original version on PR Newswire, visit:http://www.prnewswire.com/news-releases/neurocrine-biosciences-announces-expansion-of-its-clinical-pipeline-300007448.html

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Neurocrine Biosciences, Investor Relations, (858) 617-7600