



## Neurocrine Biosciences Announces Granting of Orphan Drug Status for NBI-77860 in Congenital Adrenal Hyperplasia

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SAN DIEGO, Jan. 16, 2015 /PRNewswire/ -- Neurocrine Biosciences, Inc. (NASDAQ: NBIX) announced today that NBI-77860, a proprietary corticotropin releasing factor 1 (CRF) receptor antagonist, has been granted orphan drug status by the United States Food and Drug Administration (FDA) for the treatment of congenital adrenal hyperplasia (CAH) a disease that affects approximately 20,000-30,000 people in the United States.

"We are very pleased that the FDA has granted NBI-77860 orphan status to treat congenital adrenal hyperplasia, a devastating disease that is a significant challenge for both clinicians and patients," said Malcolm Lloyd-Smith, Chief Regulatory Officer of Neurocrine Biosciences. "This status represents a significant regulatory milestone for the CAH program and underscores the importance of bringing a safe and effective CAH therapy to market. We look forward to the results from our recently initiated 1401 Study of adolescents with classic CAH, in 2015."

### About Orphan Drug Designation

Orphan Drug Designation is granted by the FDA Orphan Drug Designation program for medicines intended for the safe and effective treatment, diagnosis or prevention of rare diseases or disorders that affect fewer than 200,000 people in the United States. Orphan Drug Designation provides sponsors with development and commercial incentives for such designated compounds and medicines.

### 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 classic 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.

### 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 day after dosing.

### 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, and 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. 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-granting-of-orphan-drug-status-for-nbi-77860-in-congenital-adrenal-hyperplasia-300021863.html>

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