January 20, 2017

Concert Pharmaceuticals Receives FDA Orphan Drug Designation for CTP-656 for the Treatment of Cystic Fibrosis

LEXINGTON, Mass.--(BUSINESS WIRE)-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for CTP-656, Concert's next generation CFTR potentiator being developed for the treatment of cystic fibrosis. In December 2016, Concert initiated a Phase 2 trial in the U.S. evaluating CTP-656 in cystic fibrosis patients with gating mutations. Topline results from the Phase 2 trial are expected by year-end 2017.

"Receiving orphan drug designation is an important regulatory milestone, and we are pleased that CTP-656 for cystic fibrosis has been granted this status," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "We are developing CTP-656 to potentially offer advantages over standard of care, and our team is committed to advancing the clinical development program to address the unmet needs of individuals with cystic fibrosis."

The Orphan Drug Act provides incentives for companies to develop products for rare diseases affecting fewer than 200,000 people in the United States. Incentives may include tax credits related to clinical trial expenses, an exemption from the FDA user fee, FDA assistance in clinical trial design and potential market exclusivity for seven years following approval.

About CTP-656 and Cystic Fibrosis

CTP-656 is a novel CFTR potentiator that may offer next generation, once-daily dosing and was developed by Concert's novel application of deuterium chemistry to modify ivacaftor. Ivacaftor is marketed by Vertex Pharmaceuticals under the brand name Kalydeco. Concert is initially developing CTP-656 as a potential monotherapy treatment for cystic fibrosis due to gating mutations of the gene that encodes for cystic fibrosis transmembrane conductance regulator (CFTR), a protein, which regulates components of sweat, mucus clearance and digestion. The Company also intends to enable potentially more effective combinations to treat other mutations, including homozygous F508del, by partnering with other potentially complementary CFTR modulators.

Cystic fibrosis is a life-threatening, hereditary genetic disease that has systemic effects and can cause significantly reduced lung and digestive system function. According to the Cystic Fibrosis Foundation, an estimated 70,000 people worldwide have cystic fibrosis.

About Concert

Concert Pharmaceuticals is a clinical stage biopharmaceutical company focused on applying its DCE Platform® (deuterated chemical entity platform) to create novel medicines designed to address unmet patient needs. The Company's approach starts with approved drugs in which deuterium substitution has the potential to enhance clinical safety, tolerability or efficacy. Concert has a broad pipeline of innovative medicines targeting pulmonary diseases, including cystic fibrosis, central nervous systems (CNS) disorders, as well as autoimmune and inflammatory diseases. For more information please visit www.concertpharma.com.

Cautionary Note on Forward Looking Statements

Any statements in this press release about our future expectations, plans and prospects, including statements about clinical development of CTP-656 and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: availability and timing of data from ongoing and future clinical trials and the results of such trials, whether preliminary results from a clinical trial will be predictive of the final results of that trial or whether results of early clinical trials will be indicative of the results of later clinical trials, expectations for regulatory approvals, whether orphan drug status will be granted and other factors discussed in the "Risk Factors" section of our most recent Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission and in other filings that we make with the Securities and Exchange Commission. In addition, any forward-looking statements included in this press release represent our views only as of the date of this release and should not be relied upon as representing our views as of any subsequent date. We specifically disclaim any obligation to update any forward-looking statements included in this press release.

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Concert Pharmaceuticals, Inc.
Justine E. Koenigsberg (Investors), 781-674-5284
ir@concertpharma.com
or
The Yates Network
Kathryn Morris (Media), 845-635-9828

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