December 21, 2016

Concert Pharmaceuticals Initiates Phase 2 Clinical Trial Evaluating CTP-656 for the Treatment of Cystic Fibrosis

LEXINGTON, Mass.--(BUSINESS WIRE)-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced the initiation of a U.S.-based Phase 2 clinical trial evaluating CTP-656 (deuterated ivacaftor), a next generation CFTR potentiator being developed for the treatment of cystic fibrosis. CTP-656 was created based on Concert's application of deuterium chemistry to modify ivacaftor, which is commercially available under the brand name Kalydeco® and is the current standard of care for cystic fibrosis patients with gating mutations. Topline results of the Phase 2 trial of CTP-656 are expected by year-end 2017.

"We are excited to initiate our Phase 2 trial with CTP-656 in individuals with cystic fibrosis," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "In contrast to Kalydeco, Phase 1 clinical results from CTP-656 support dosing it once-daily with food, without regard to the fat content of the food, potentially enabling CTP-656 to support improved adherence and provide real world benefits for patients with cystic fibrosis."

The Phase 2 clinical trial is a randomized, parallel-group, double-blind, placebo-controlled, clinical trial to evaluate the safety and efficacy of CTP-656 in cystic fibrosis patients with gating mutations. Patients enrolled in the 28-day study will receive one of three doses (20 mg, 100 mg and 150 mg) of CTP-656 once-daily or placebo. There will also be an open-label Kalydeco comparator arm in the trial. Approximately 30-40 patients will be enrolled in the Phase 2 trial which will be conducted in multiple centers in the U.S. The primary endpoint of the Phase 2 trial is a change from baseline in sweat chloride at Day 28. Secondary endpoints include change in percent predicted forced expiratory volume (FEV_1) and change from baseline in CFQ-R Respiratory Domain. The study is being conducted within the Cystic Fibrosis Foundation's Therapeutic Development Network (TDN). Additional information about the Phase 2 clinical trial is available at www.clinicaltrials.gov.

In Phase 1 clinical evaluations, CTP-656 provided superior key exposure parameters compared to Kalydeco. CTP-656 was well-tolerated and its safety profile was comparable to that of Kalydeco. No serious adverse events were reported in Phase 1 studies.

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 and outcomes 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: the uncertainties inherent in the initiation of future clinical trials, 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 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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