January 17, 2017

Concert Pharmaceuticals Provides Further Details on CTP-656 Development in U.S. and Europe

Company to Host Conference Call and Webcast Today, January 17, 2017 at 8:30 A.M. ET

LEXINGTON, Mass.--(BUSINESS WIRE)-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today provided further details on the development plan in the U.S. and Europe for CTP-656, a next generation CFTR potentiator being developed for the treatment of cystic fibrosis.

In the U.S., Concert initiated a placebo-controlled Phase 2 trial in December 2016 to evaluate CTP-656 in cystic fibrosis patients with gating mutations. Subsequent to the initiation of the study, the U.S. Food and Drug Administration (FDA) informed Concert that, in order to support dose selection for Phase 3, an adequate washout period, in which Kalydeco® (ivacaftor) treatment is withheld, would be required in addition to a placebo-control. The Company intends to further discuss the additional feedback with FDA. The ongoing Phase 2 trial is being conducted at multiple U.S. study sites within the Cystic Fibrosis Foundation's Therapeutic Development Network with topline data expected by year-end 2017.

In Europe, Concert intends to initiate an open-label Phase 2 trial in the second quarter of 2017. The trial is expected to enroll 14 cystic fibrosis patients with gating mutations stable on Kalydeco. Patients will be switched to one dose of CTP-656 for two weeks followed by a switch to a higher dose of CTP-656 for an additional two weeks. Subsequently, patients will resume treatment with Kalydeco. The primary analysis will be for non-inferiority for sweat chloride compared to the Kalydeco baseline. Topline data from the European Phase 2 study of CTP-656 is expected by year-end 2017.

"We are entering a new stage in which the next wave of drug candidates for cystic fibrosis - beyond today's standard of care - are advancing into later-stage clinical development. We look forward to further discussions with FDA on the development of CTP-656 and believe that our ongoing U.S. trial together with the planned European study will yield data to support the progression of CTP-656," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "We look forward to the continued advancement of CTP-656 for both monotherapy and potential future combination therapy."

Conference Call and Webcast

The Company will host a conference call and webcast today at 8:30 a.m. EST to discuss CTP-656. To access the conference call, please dial (855) 354-1855 (U.S. and Canada) or (484) 365-2865 (International) five minutes prior to the start time.

A live webcast of Concert's presentation may be accessed in the Investors section of the Company's website at www.concertpharma.com. Please log on to the Concert website approximately 15 minutes prior to the scheduled webcast to ensure adequate time for any software downloads that may be required. A replay of the webcast will be available on Concert's website for two weeks.

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 about the 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, our ability to reach agreement with the FDA regarding an adequate washout period to support dose selection for Phase 3 and our ability to recruit patients for any such trial, 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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