October 27, 2016

Concert Pharmaceuticals Presents Poster at 2016 North American Cystic Fibrosis Conference

Clinical Results Demonstrated CTP-656's Features as Potential Next Generation, Once-Daily CFTR Potentiator for Treatment of Cystic Fibrosis

LEXINGTON, Mass.--(BUSINESS WIRE)-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today presented a poster describing Phase 1 food effect and multiple ascending dose results of CTP-656 at the North American Cystic Fibrosis Conference being held in Orlando, Florida. CTP-656 is a next generation CFTR potentiator being developed for the treatment of cystic fibrosis. The food effect results showed that the exposure of CTP-656 was the same regardless of the fat content of a meal. In addition, the safety, tolerability and pharmacokinetic profile of CTP-656 observed to date supports its development as a once-daily CFTR potentiator. The Company intends to open an Investigational New Drug Application (IND) with the U.S. Food and Drug Administration (FDA) by year-end 2016 to enable the initiation of its Phase 2 clinical trial. Topline results from the Phase 2 trial are expected in the second half of 2017.

"The Phase 1 results with CTP-656 demonstrated a superior pharmacokinetic profile relative to Kalydeco®, the current standard of care. We believe this profile may result in a number of potential advantages over current treatments including, simplified dosing, enhanced efficacy and reduced drug-drug interaction," said James Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. "We are working expeditiously to advance CTP-656 into our U.S. Phase 2 trial in 2017."

The Phase 1 food-effect study was conducted in 15 healthy volunteers to evaluate the relative bioavailability of CTP-656 under fasted and fed conditions, with low (approximately 7 grams) and moderate (approximately 20 grams) amounts of fat, in order to assess food requirements for administration of CTP-656. Kalydeco is required to be taken with fat containing foods every 12 hours. CTP-656 exposure with low-fat and moderate-fat food conditions were similar to those observed in a previous Phase 1 single dose crossover study conducted under high-fat (approximately 60 grams) conditions.

"The study findings support the potential to simply take CTP-656 with food, providing substantial dosing flexibility to patients," said Dr. Cassella.

Clinical highlights from the food-effect study include:

- The mean Cmax, AUC and C24hr for CTP-656 under both fed conditions was approximately 2-fold greater than under fasted conditions;
- The mean Cmax, AUC and C24hr for the major metabolite was approximately 1.7-fold higher in the fed versus fasted conditions; and
- CTP-656 was well-tolerated under all conditions.

The multiple ascending dose Phase 1 trial assessed safety, tolerability and pharmacokinetics of CTP-656 in a tablet formulation in 38 healthy volunteers. Three doses were assessed (75 mg, 150 mg and 225 mg) for seven days compared to placebo. The study also assessed a single dose pharmacokinetic comparison of 150 mg solid dose tablet of CTP-656 compared to 150 mg of Kalydeco. The study results showed that CTP-656 provided substantially superior key exposure parameters compared to Kalydeco. In addition, CTP-656 maintained its superior pharmacokinetic profile with greater exposure to the more potent parent drug than to less active metabolites. The Phase 1 multiple ascending dose results were presented in an oral presentation at the 2016 European Cystic Fibrosis Conference earlier this year.

In the Phase 1 studies, CTP-656 was well-tolerated and its safety profile was comparable to that of Kalydeco. No serious adverse events were reported.

A copy of the poster is available online at: http://www.concertpharma.com/technology-overview/presentations/

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. 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: 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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