March 4, 2017

Concert Pharmaceuticals Presents CTP-543 Phase 1 Results at American Academy of Dermatology Annual Meeting

- Program on Track to Begin Phase 2a Trial in the First Quarter of 2017 -

LEXINGTON, Mass.--(BUSINESS WIRE)-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced that the results from the Company's Phase 1 single and multiple ascending dose trial evaluating CTP-543 in healthy volunteers were presented in a poster session at the American Academy of Dermatology's Annual Meeting in Orlando, FL. Concert is developing CTP-543 for the treatment of moderate-to-severe alopecia areata, an autoimmune disorder in which the immune system attacks hair follicles, resulting in patchy or complete hair loss. CTP-543 is a deuterium-modified analog of ruxolitinib, a drug which is commercially available in the U.S. under the brand name Jakafi® for the treatment of certain blood disorders. The results of the Phase 1 trial support the further evaluation of CTP-543 in patients with alopecia areata. Concert intends to initiate a Phase 2a trial in the first quarter of 2017, with topline results expected by year-end 2017.

"We are highly encouraged by the favorable safety and pharmacokinetic profile of CTP-543 observed in Phase 1, and we believe CTP-543 has the potential to become an important treatment option for individuals with moderate-to-severe alopecia areata," said James Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. "There is a significant need to develop an effective treatment for alopecia areata and Concert is at the forefront with CTP-543."

The Phase 1 program was designed to determine the safety, tolerability and pharmacokinetics of CTP-543 in a total of 77 healthy volunteers. In the single ascending dose portion of the program, oral doses of 8, 16, 32 and 48 mg CTP-543 were evaluated. For the multiple ascending dose portion, escalating doses of CTP-543 up to 32 mg daily (studied both as once-daily 32 mg doses and as twice-daily 16 mg doses) were administered for seven consecutive days.

In Phase 1, CTP-543 was well tolerated across all dose groups and there were no serious adverse events reported in the study. The most common reported adverse event was headache. There were no withdrawals or dose modifications related to CTP-543 in the trial.

In the trial, CTP-543 showed increased exposure with increasing doses. The safety and exposure observed with 16 mg of CTP-543 twice daily appeared to be comparable to published findings for the reported exposure of 20 mg ruxolitinib twice daily. CTP-543 had a half-life of approximately 3.3 hours, similar to that reported for non-deuterated ruxolitinib[1]. In an open-label clinical trial of 12 patients with moderate-to-severe alopecia areata, investigators at Columbia University reported that 20 mg of ruxolitinib administered orally twice daily resulted in significant hair regrowth in 75% of the patients[2]. Given the overall exposure and safety parameters observed with CTP 543 in the Phase 1 program, the Company has selected four doses (4, 8, 12 and 16 mg twice daily) to evaluate in its double-blind, placebo-controlled Phase 2a trial.

A copy of the poster presented at the American Academy of Dermatology's Annual Meeting is available on Concert's website at http://www.concertpharma.com/technology-overview/presentations/.

About CTP-543 and About Alopecia Areata
CTP-543 was discovered by applying Concert's deuterium chemistry technology to modify ruxolitinib, which is commercially available under the name Jakafi® in the United States for the treatment of certain blood disorders. Ruxolitinib has been used to treat alopecia areata in academic settings, including an investigator-sponsored clinical trial, and has been shown to promote hair growth in individuals with moderate to severe disease.

Alopecia areata is an autoimmune disease that results in partial or complete loss of hair on the scalp and body that may affect up to 650,000 Americans at any given time[3]. The scalp is the most commonly affected area, but any hair-bearing site can be affected alone or together with the scalp. Onset of the disease can occur throughout life and affects both women and men. Alopecia areata can be associated with serious psychological consequences, including anxiety and depression. There are currently no drugs approved by the U.S. Food and Drug Administration (FDA) for the treatment of alopecia areata.

In 2015, the FDA selected alopecia areata as one of eight new disease areas that it will focus on under its Patient-Focused Drug Development Initiative (PFDDI) meeting during fiscal 2016-2017. The goal of the PFDDI is to bring patient perspectives into an earlier stage of product development.

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, autoimmune and inflammatory diseases and central nervous systems (CNS) disorders. 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 our expectations on the clinical development of CTP-543, 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, 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 Annual Report on Form 10-K 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.

References:
[1] Shi et al, J Clin Pharmacol, 2011.

[2] JCI Insight. 2016;1(15):e89790. doi:10.1172/jci.insight.89790.

[3] Fricke M. Epidemiology and Burden of alopecia areata: a systemic review. Clinical, Cosmetic and Investigational Dermatology. 2015; Vol 8. 397-403.

View source version on businesswire.com: http://www.businesswire.com/news/home/20170304005003/en/

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

Source: Concert Pharmaceuticals, Inc.