# Concert Pharmaceuticals Amends Protocol of Phase 2a Trial to Evaluate 12 mg Twice-Daily Dose Cohort of CTP-543 for the Treatment of Alopecia Areata

September 26, 2018

***Company On Track to Report Topline Data from the 4 mg and 8 mg Twice-Daily Cohorts in Fourth Quarter of 2018***

LEXINGTON, Mass.--(BUSINESS WIRE)--Sep. 26, 2018-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced that it will include an additional cohort of patients in the Phase 2a trial evaluating CTP-543. The protocol amendment provides for additional patients to be enrolled in the trial in order to evaluate a 12 mg dose of CTP-543 or placebo twice daily for 24 weeks. In the third quarter of 2018, an independent Data Monitoring Committee (DMC) conducted a planned interim safety data review after patients in the trial had been dosed with an 8 mg dose of CTP-543 or placebo twice daily for at least 12 weeks. Based on this review, the DMC provided its recommendation to continue with the current 8 mg cohort to completion and also provided support for an additional cohort to evaluate the 12 mg dose twice daily. The Company has initiated enrollment in the 12 mg cohort. The Company also expects to report topline data from the 4 mg and 8 mg cohorts of the Phase 2a trial in the fourth quarter of 2018.

“We believe that understanding a broader dose range in the Phase 2a trial will be important to our selection of the CTP-543 doses for late stage clinical development,” stated James Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. “We are grateful for the patient community’s support of the CTP-543 development program, as we work at the forefront of exploring new treatment options for patients with our oral JAK inhibitor for the treatment of alopecia areata.”

The Phase 2a trial is a double-blind, randomized, placebo-controlled, sequential dose trial to evaluate the safety and efficacy of CTP-543 in adult patients with moderate-to-severe alopecia areata. With the protocol amendment, patients are randomized to receive one of three doses of CTP-543 (4 mg, 8 mg, or 12 mg) or placebo twice daily. Enrollment in the 4 mg and 8 mg cohorts is complete and enrollment in the 12 mg cohort is ongoing. The primary outcome measure will utilize the severity of alopecia tool (SALT) after 24 weeks of dosing. Additional information about the trial is available on www.clinicaltrials.gov.

**About CTP-543 and Alopecia Areata**

CTP-543 was discovered by applying Concert's deuterium chemistry technology to modify ruxolitinib, a drug which selectively inhibits Janus kinases 1 and 2 (JAK1 and JAK2) and 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 reported to promote hair growth in individuals with moderate-to-severe disease. Deuterium modification of ruxolitinib was found to alter its human pharmacokinetics in ways which may enhance its use as a treatment for alopecia areata. The U.S. Food and Drug Administration (FDA) has granted Fast Track designation for CTP-543.

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[1]. 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.

The FDA selected alopecia areata as one of eight new disease areas that it focused on under its Patient-Focused Drug Development Initiative (PFDDI) in 2016-2017. The goal of the PFDDI is to bring patient perspectives into an earlier stage of product development. Following the U.S. Food and Drug Administration’s ( FDA) Patient-Focused Drug Development meeting held in September 2017 on alopecia areata, the FDA summarized the input shared by patients and patient representatives in a Voice of the Patient report. Additional information on the PFDDI is available online.

**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 treat serious diseases and address unmet patient needs. The Company’s approach starts with previously studied compounds, including 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 autoimmune and inflammatory diseases and central nervous systems (CNS) disorders. For more information please visit www.concertpharma.com or follow us on Twitter at @ConcertPharma or on LinkedIn.

**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 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.

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

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