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Concert Pharmaceuticals Announces Completion of Enrollment in First Cohort of CTP-543 Phase 2a Trial

CTP-543 is Being Evaluated in Moderate-to-Severe Alopecia Areata

Phase 2a Trial On Track to Complete in Second Half of 2018

LEXINGTON, Mass.--(BUSINESS WIRE)-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced that it has completed enrollment of the first cohort of its Phase 2a trial evaluating CTP-543. 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 Janus Kinase (JAK) inhibitor that is commercially available under the brand name Jakafi® for the treatment of certain blood disorders.

In the Phase 2a trial, patients with alopecia areata in the first cohort are administered 4 mg of CTP-543 or placebo twice daily for 24 weeks. An independent Data Monitoring Committee (DMC) will conduct an interim safety data review from the first cohort after patients have completed three months of dosing. This review is expected to be completed in the first quarter of 2018. Based on this review, the DMC will provide its recommendation regarding initiating the second cohort in the trial, which will be administered 8 mg of CTP-543 or placebo twice daily for 24 weeks. The Phase 2a trial is expected to be completed in the second half of 2018.

"We are excited to see the enthusiastic participation by alopecia areata patients in our study. Its sequential dosing design will allow us to assess the safety and efficacy of CTP-543 in this disease in a controlled and deliberate manner," said James Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. "We look forward to the DMC's assessment, and we are on track with our goal to complete the trial in the second half of 2018."

The Phase 2a trial is a double-blind, randomized, placebo-controlled trial to evaluate the safety and efficacy of CTP-543 in adults with moderate-to-severe alopecia areata. Approximately 90 patients are being enrolled in the study and sequentially randomized to receive one of two doses of CTP-543 (4 or 8 mg) or placebo twice daily. The primary outcome measure will utilize the severity of alopecia tool (SALT) after 24 weeks of dosing. Patient-reported outcome measures will be assessed as secondary endpoints. If appropriate, the protocol may be amended to explore higher doses of CTP-543. Additional information about the trial is available on www.clinicaltrials.gov.

CTP-543 has been well-tolerated in clinical evaluation to date and has demonstrated a non-clinical safety profile consistent with therapeutic JAK inhibition.

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.

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) meeting in 2016-2017. The goal of the PFDDI is to bring patient perspectives into an earlier stage of product development. The meeting was held on Monday, September 11, 2017. Additional information is available online at: https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm554443.htm

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 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 for 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, availability of funding sufficient for our foreseeable and unforeseeable operating expenses and capital expenditure requirements 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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