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FDA Lifts Clinical Hold on Concert Pharmaceuticals Clinical Trial with CTP-543

Concert to Resume Enrollment in Modified Phase 2a Trial for Alopecia Areata

LEXINGTON, Mass.--(BUSINESS WIRE)-- Concert Pharmaceuticals, Inc. (NASDAQ:CNCE) today announced that the U.S. Food and Drug Administration (FDA) has notified the Company that it has lifted the clinical hold on Concert's CTP-543 Phase 2a clinical trial for alopecia areata. The Company will amend the protocol for the Phase 2a trial to evaluate two doses of CTP-543, each for a 24 week duration. Concert will resume enrollment in the Phase 2a trial later this month and expects to complete the trial in the second half of 2018.

"We believe the FDA is taking a measured approach for the development of Janus kinases inhibitors, or JAK inhibitors, for autoimmune dermatological disorders, including alopecia areata. Accordingly, we agreed to modify the design of our Phase 2a trial to evaluate CTP-543 in the patient population in a more step-wise fashion. It is important to note that CTP-543 has been well-tolerated in clinical evaluation to date and demonstrates a non-clinical safety profile consistent with JAK inhibition," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "Alopecia areata is an autoimmune disease for which there is a significant unmet medical need with no FDA-approved therapy, and we intend to be at the forefront of advancing a new oral treatment for alopecia areata patients."

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 will be enrolled in the study and will be sequentially randomized to receive one of two doses of CTP-543 (4 and 8 mg twice daily) or placebo. The primary outcome measure will utilize the severity of alopecia tool (SALT) after 24 weeks of dosing. 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.

In May 2017, the FDA informed the Company that a review of certain non-clinical toxicology studies was required before proceeding with dosing in the Phase 2a trial. Concert submitted the requested non-clinical study reports and in further discussions with FDA agreed to amend the clinical protocol leading to today's announcement of the FDA's lifting of the clinical hold. In non-clinical evaluation, the profile of CTP-543 was as expected given the compound's mechanism of action.

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 will focus on under its Patient-Focused Drug Development Initiative (PFDDI) meeting during fiscal year 2016-2017. The goal of the PFDDI is to bring patient perspectives into an earlier stage of product development. The meeting is scheduled for 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 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 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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