Concert Pharmaceuticals Completes Enrollment in Open Label Trial to Evaluate Once-Daily vs. Twice-Daily Dosing of CTP-543 in Patients with Alopecia Areata

June 5, 2019

LEXINGTON, Mass.--(BUSINESS WIRE)--Jun. 5, 2019-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced that it has completed patient enrollment of an open label clinical trial to evaluate once-daily compared to twice-daily oral dosing of CTP-543 in patients with alopecia areata. The trial is designed to inform on the optimal dosing regimen for CTP-543 for future clinical trials. Topline data from the dose regimen trial is expected in the fourth quarter of 2019.

“This dose regimen trial helps us understand the safety and efficacy of CTP-543 when dosed once daily and allows us to set our dosing strategy for future late-stage trials. We are grateful to the patients who participated in this and other trials, as we pursue a new treatment for alopecia areata, a disease that currently has no FDA-approved treatments,” said James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals.

The open label trial (NCT03811912) is a randomized, multi-center study to evaluate the efficacy and tolerability of once-daily versus twice-daily dosing of CTP-543, in 57 adult patients with chronic, moderate-to-severe alopecia areata. Patients in the trial were randomized to receive CTP-543 either 8 mg twice-daily or 16 mg once-daily over a 24 week treatment period. The trial will measure the relative change in Severity of Alopecia Tool (SALT) score between Week 24 and baseline. All patients who complete 24 weeks of treatment will be eligible to enroll into an extension study of CTP-543.

A second open label trial (NCT03941548) to evaluate once-daily compared to twice-daily oral dosing was initiated in May 2019 and is currently enrolling patients. Patients in the trial will be randomized to receive either 12 mg twice-daily or 24 mg once-daily of CTP-543 over a 24 week treatment period. All patients who complete 24 weeks of treatment will be eligible to enroll into an extension study of CTP-543.

Additional information about CTP-543 clinical trials are available on www.clinicaltrials.gov.

About CTP-543 Interim Phase 2 Results

During the late-breaking clinical trials session at the American Academy of Dermatology (AAD) Annual Meeting on March 2, 2019, the Company presented interim results from an ongoing Phase 2 trial. At 24 weeks, patients treated with an 8 mg twice-daily dose of CTP-543 met the primary efficacy endpoint with a statistically significant difference compared to placebo ($p < 0.001$). The primary outcome measure was the proportion of patients with a ≥ 50% relative reduction in their overall SALT score between Week 24 and baseline. As reported, CTP-543 was generally well tolerated and there were no serious adverse events reported. Dosing in an additional cohort receiving a 12 mg twice-daily dose of CTP-543 compared to placebo in the Phase 2 trial is currently ongoing. Additional information about the CTP-543 Phase 2 results is available in the Scientific Presentations section of Company’s website.

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. 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 with the majority of patients initially having symptoms by age 40. It is believed to equally affect 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's pipeline of innovative medicines currently targets autoimmune 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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