Concert Pharmaceuticals Reports Positive CTP-543 Results from Interim Analysis of Phase 2a Trial in Patients with Alopecia Areata

November 12, 2018

CTP-543 Achieved its Primary Endpoint in the 8 mg Twice-Daily Cohort

Company to Host Investor Conference Call Today at 8:30 a.m. ET

LEXINGTON, Mass.--(BUSINESS WIRE)--Nov. 12, 2018-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced interim topline results from the first two cohorts of its Phase 2a trial evaluating its investigational treatment CTP-543 in patients with moderate-to-severe alopecia areata, an autoimmune disorder in which the immune system attacks hair follicles, resulting in patchy or complete hair loss. At 24 weeks, patients treated with an 8 mg twice-daily dose of CTP-543 met the primary efficacy endpoint vs. placebo ($p < 0.001$). At this dose, significant differences from placebo were observed beginning at Week 12. Regrowth of hair did not appear to plateau at Week 24. Treatment with CTP-543 was generally well tolerated. Dosing in a 12 mg twice-daily cohort is currently underway. Complete study results are expected to be presented at a future medical meeting.

"We are very pleased by these initial efficacy and tolerability data for CTP-543 in patients with moderate-to-severe alopecia areata. We believe that the extent of efficacy and tolerability of CTP-543 to date supports its further development," said James Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. "We look forward to completing the final dosing cohort in this Phase 2a trial and advancing the program into later stage clinical trials."

The primary efficacy endpoint in the 8 mg twice-daily cohort was met with 47% of patients achieving a ≥ 50% relative reduction in their overall severity of alopecia tool (SALT) score from baseline compared to placebo ($p < 0.001$). For the 4 mg cohort, 21% of patients achieved a ≥ 50% relative reduction in their overall SALT score from baseline, however these differences were not significantly different from placebo. In the primary analysis, the response observed in the 8 mg twice-daily dose was significantly different than the 4 mg twice daily dose ($p < 0.05$). The average baseline SALT score across all patients enrolled in the trial was approximately 88.

Compared to placebo, a significant relative reduction in mean SALT score was first observed in the 8 mg cohort at Week 12 ($p < 0.05$).

The most common side effects in the trial were headache, upper respiratory tract infection, cough, acne and nausea. No serious adverse events were reported.

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. Patients will be randomized to receive one of three doses of CTP-543 (4 mg, 8 mg, or 12 mg) or placebo twice-daily. The final cohort evaluating a 12 mg twice-daily dose of CTP-543 compared to placebo is ongoing. The primary outcome measure will utilize the severity of alopecia tool (SALT) after 24 weeks of dosing. Results from the complete Phase 2a trial, including the 12 mg cohort, are expected in the third quarter of 2019.

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 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.

Conference Call and Webcast

The Company will host a conference call and webcast today at 8:30 a.m. ET to discuss the CTP-543 Phase 2a topline results. To access the conference call, please dial (855) 354-1855 (U.S. and Canada) or (484) 365-2865 (International) five minutes prior to the start time.

A live webcast of Concert's presentation may be accessed in the Investors section of the Company's website at www.concertpharma.com. Please log on to the Concert website approximately 15 minutes prior to the scheduled webcast to ensure adequate time for any software downloads that may be required. A replay of the webcast will be available on Concert's website for three months.

A corresponding slide presentation to accompany the conference call is available in the Scientific Presentations section of Company's website.

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 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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