Concert Pharmaceuticals Reports Positive CTP-543 Results from Phase 2 Alopecia Areata Trial

September 3, 2019

CTP-543 Achieved its Primary Endpoint in the 8 mg and 12 mg Twice-Daily Dosing Cohorts

Advancement into Phase 3 Evaluation Planned in 2020

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

LEXINGTON, Mass.--(BUSINESS WIRE)--Sep. 3, 2019-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced final topline results from its recently completed dose-ranging Phase 2 trial evaluating its investigational medicine CTP-543 in patients with moderate-to-severe alopecia areata, an autoimmune disorder that results in patchy or complete hair loss. Patients treated with either 8 mg twice-daily or 12 mg twice-daily of CTP-543 met the primary efficacy endpoint with statistically significant differences ($p < 0.001$) relative to placebo in the percentage of patients achieving a ≥ 50% relative change from baseline at 24 weeks using the Severity of Alopecia Tool (SALT). The 8 mg twice-daily and 12 mg twice-daily groups were also significantly different from placebo in the number of patients achieving ≥ 75% and ≥ 90% relative change in SALT from baseline at 24 weeks. At Week 24, patients treated with 8 mg twice-daily and 12 mg twice-daily compared to placebo also rated significantly greater improvement in their alopecia areata on the Patient Global Impression of Improvement Scale. Treatment with CTP-543 was generally well tolerated. Complete study results are expected to be presented at a future medical meeting.

“We are very pleased with these clinical results and continue to believe CTP-543 has potential to be a best-in-class treatment for alopecia areata, a chronic dermatological autoimmune disease that currently has no approved therapies,” said James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. “We are highly focused on the need for an effective and safe treatment for alopecia areata, and we plan to advance CTP-543 into Phase 3 testing next year.”

“These Phase 2 results of CTP-543, a Janus kinase or JAK inhibitor, for the treatment of patients with alopecia areata are highly encouraging,” stated Dr. Brett King, Associate Professor of Dermatology at Yale School of Medicine. “There is a growing body of evidence supporting JAKs as a target for the treatment of alopecia areata that is driving enthusiasm in dermatology to address an important unmet need for patients.”

The primary efficacy endpoint in the 12 mg twice-daily cohort was met with 58% of patients achieving a ≥ 50% relative reduction in their overall SALT score from baseline compared to 9% for placebo ($p < 0.001$). In the 8 mg twice-daily cohort, 47% of patients achieved the primary endpoint 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 addition, 78% of the patients receiving 12 mg twice-daily and 58% of the patients receiving 8 mg twice-daily rated their alopecia areata as “much improved” or “very much improved” at 24 weeks of dosing, a significant difference from placebo.

The average baseline SALT score across all patients was approximately 88%, where 0% is no scalp hair loss and 100% represents total scalp hair loss. The most common (≥10%) side effects in the 12 mg CTP-543 dose group were headache, nasopharyngitis, upper respiratory tract infection, and acne. One serious adverse event of facial cellulitis was reported as possibly related to treatment; however, after a brief interruption, treatment was continued and this patient completed the trial. No thromboembolic events were reported during the trial.

The Phase 2 trial was 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. A total of 149 patients were randomized to receive one of three doses of CTP-543 (4 mg, 8 mg, or 12 mg) or placebo, administered twice-daily. The primary outcome measure utilized the SALT score after 24 weeks of dosing. All patients who completed 24 weeks of treatment in the 12 mg dosing cohort had the opportunity to continue in a separate extension study to evaluate long-term safety and efficacy of CTP-543.

About CTP-543 and Alopecia Areata

CTP-543 is an oral selective inhibitor of Janus kinases JAK 1 and JAK 2 for the potential treatment of alopecia areata. The U.S. Food and Drug Administration (FDA) has granted Fast Track designation for CTP-543.

Alopecia areata is an autoimmune disease in which the immune system attacks hair follicles, resulting in partial or complete loss of hair on the scalp and body. Alopecia areata 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 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 FDA’s 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 2 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's pipeline of innovative medicines 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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