Concert Pharmaceuticals Releases Additional Data from Phase 2 Clinical Trial of CTP-543 in Alopecia Areata

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New Analyses Show Statistically Significant Differences from Placebo for the 8 mg and 12 mg Twice-Daily Cohorts Achieving a SALT Score of ≤ 20

CTP-543 Expected to Advance into Phase 3 Evaluation in the Fourth Quarter of 2020

LEXINGTON, Mass.--(BUSINESS WIRE)--Jun. 12, 2020-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today released new data analyses from its Phase 2 dose-ranging clinical trial of its investigational agent CTP-543 for the treatment of moderate-to-severe alopecia areata. The results were selected as an oral presentation at the Late-Breaking Research Program at the American Academy of Dermatology (AAD) Annual Meeting; however, the in-person meeting, originally scheduled for March 21, 2020, in Denver, was converted to a virtual meeting. The corresponding abstract was published by AAD in connection with the virtual meeting. The data analyses released by Concert build on the previously-reported Phase 2 primary efficacy analysis which showed that administration of 8 mg twice-daily and 12 mg twice-daily doses of CTP-543 for 24 weeks produced a statistically significantly greater number of responders compared to placebo. A responder was defined as a ≥ 50% relative reduction in Severity of Alopecia Tool (SALT) score at Week 24 compared to baseline. In the new analyses, statistically significant results were reported for the 8 mg and 12 mg twice-daily doses at more stringent response thresholds, which may be more clinically meaningful to patients, and positive findings were reported for clinician and patient reported outcome measures of scalp hair loss.

"We believe that these data from our Phase 2 dose-ranging study set a new benchmark for clinical efficacy in treating alopecia areata and provide important information to inform our planned Phase 3 trials with CTP-543. Since both the 8 mg and 12 mg twice-daily doses resulted in statistically significant improvement in SALT scores compared to placebo and exhibited a generally well-tolerated safety profile, we plan to assess both doses in our Phase 3 program," said James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. "Importantly, we would like to thank the alopecia areata patients for their participation in this study, as well as the clinical investigators and research staff for their significant efforts to support the development of CTP-543."

Key new findings further highlight the positive efficacy results in the Phase 2 study, including:

- **Meaningful hair regrowth:** At Week 24, 26% and 42% of patients who received CTP-543 in the 8 and 12 mg twice-daily cohorts, respectively, achieved an absolute SALT score ≤ 20 ($p < 0.05$ vs. placebo), indicating a clinically-meaningful 80 percent or greater scalp hair present. In addition, 36% of patients in the 12 mg twice-daily cohort achieved an absolute SALT score ≤ 10 ($p < 0.05$ vs. placebo) at Week 24.
- **Clinical Global Impression of Improvement:** Data from the Clinician Global Impression of Improvement scale showed 75% of clinicians rated the response in the 12 mg twice-daily cohort and 61% of clinicians rated the response in the 8 mg twice-daily cohort as "much improved" or "very much improved" at Week 24. For both doses there was a statistically significant difference from placebo ($p < 0.001$).
    - Similarly, previously-reported data from the Phase 2 trial for the Patient Global Impression of Improvement scale showed 78% of patients receiving 12 mg twice-daily and 58% of patients receiving 8 mg twice-daily rated their alopecia areata as "much improved" or "very much improved" at Week 24. For both doses there was a statistically significant difference from placebo ($p < 0.001$).

In the Phase 2 dose-ranging trial, the most common (≥10%) side effects in the 8 mg or 12 mg CTP-543 dose groups were headache, nasopharyngitis, upper respiratory tract infection, acne, nausea and low-density lipoprotein increase. One serious adverse event of facial cellulitis was reported in the 12 mg dose group as possibly related to treatment. After a brief dosing interruption, treatment was continued, and this patient completed the trial. No thromboembolic events were reported during the trial.

Slides summarizing the new analyses are available in the Scientific Presentations section of Concert's website.

About the Phase 2 Trial Design of CTP-543 in Alopecia Areata

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. 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. 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.

Previously-reported data from the Phase 2 trial demonstrated a relative reduction in overall SALT scores from baseline at Week 24, the primary efficacy endpoint of the study. In the 12 mg twice-daily cohort, 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 ($p < 0.001$).

About CTP-543 and Alopecia Areata

CTP-543 is an oral selective inhibitor of Janus kinases JAK1 and JAK2. The FDA has granted Fast Track designation for CTP-543 for the treatment of alopecia areata. The Company intends to advance CTP-543 into Phase 3 evaluation in the fourth quarter of 2020.

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 approximately 700,000 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.

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, among others, statements about our expectations on the progress of clinical development of CTP-543, and any other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "will," "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 and timing 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] Benigno M. Clinical, Cosmetic and Investigational Dermatology. 2020.

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