Concert Pharmaceuticals Presents Positive Phase 2 Data in Alopecia Areata During Late-Breaker Session at EADV Congress

October 12, 2019

LEXINGTON, Mass.--(BUSINESS WIRE)--Oct. 12, 2019-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today presented results from its recently completed Phase 2 dose-ranging trial of the investigational medicine CTP-543 in patients with moderate-to-severe alopecia areata, an autoimmune disorder that results in patchy or complete hair loss. Patients in the study 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). These results were presented during the 28th European Academy of Dermatology and Venereology (EADV) Annual Congress in Madrid, Spain.

"We are very pleased that we were selected to present our safety and efficacy data for CTP-543 to the treatment community at this Congress. We believe the results from this dose-ranging study with our oral JAK inhibitor, CTP-543, set a new bar for clinical results in patients with moderate-to-severe alopecia areata," stated James V. Cassella, Ph.D., Concert's Chief Development Officer, who made the oral presentation at EADV. "We look forward to advancing the development of CTP-543 into Phase 3 evaluation next year, as we continue our efforts to bring a new treatment to patients with this serious autoimmune disorder that currently has no approved medications."

In the Phase 2 trial, 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 ($p < 0.001$), with statistically significant separation from placebo occurring at Week 12. 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 there was not a statistically significant difference from placebo.

Data from the Patient Global Impression of Improvement scale showed 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 Week 24. For both doses there was a statistically significant difference from placebo ($p < 0.001$).

In the Phase 2 trial, 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. After a brief dosing 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. 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.

Details from the oral presentation, entitled "CTP-543, an oral JAK inhibitor, achieves primary endpoint in Phase 2 randomized, placebo-controlled, dose ranging trial in patients with moderate-to-severe alopecia areata," is available in the Scientific Presentations section of Concert's website.

About CTP-543 and Alopecia Areata

CTP-543 is an oral inhibitor of Janus kinases JAK1 and JAK2 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 was 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 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 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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