Concert Pharmaceuticals Presents Interim CTP-543 Phase 2 Data in Alopecia Areata during Late-Breaker Session at American Academy of Dermatology Annual Meeting

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Interim Data Analysis from 8 mg Twice-Daily Cohort of Investigational CTP-543 Showed Statistically Significant Effect on Primary Endpoint in Patients with Moderate-to-Severe Alopecia Areata

LEXINGTON, Mass.--(BUSINESS WIRE)--Mar. 1, 2019-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced that interim results from its Phase 2 clinical trial evaluating its product candidate CTP-543 in patients with moderate-to-severe alopecia areata will be presented in an oral presentation during the late-breaking clinical trials session at the American Academy of Dermatology (AAD) Annual Meeting on March 2, 2019, in Washington, DC. The interim results from this dose-ranging trial showed that treatment with CTP-543 administered at an 8 mg twice-daily dose for 24 weeks met the primary endpoint with a statistically significant greater hair regrowth responder rate compared to placebo. The primary endpoint measures the proportion of patients who are responders to treatment, defined as a ≥ 50% relative reduction in their overall Severity of Alopecia Tool (SALT) score from baseline.

“Alopecia areata is a chronic autoimmune disease resulting in patchy or complete hair loss that can have a profound effect on patients’ health and well-being and can impact their daily lives. I am happy to see new investigational medicines emerging with promising clinical data, including Janus kinase (JAK) inhibitors such as CTP-543,” said Dr. Brett King, Associate Professor of Dermatology at Yale School of Medicine. “The data for CTP-543 shows that JAK inhibitors have the potential to be an effective treatment option for patients with this challenging disease.”

Patients with moderate-to-severe alopecia areata enrolled in the first two cohorts received 4 mg or 8 mg of CTP-543 twice-daily or placebo twice-daily for 24 weeks. The final cohort evaluating 12 mg of CTP-543 or placebo twice-daily is ongoing. The trial randomized 104 patients in the 4 mg and 8 mg cohorts.

The oral presentation will highlight the interim results for the 8 mg twice-daily dosing cohort of CTP-543, which achieved the Phase 2 trial’s primary endpoint, in patients with moderate-to-severe alopecia areata after 24 weeks of dosing. 47% of patients treated with 8 mg of CTP-543 twice-daily achieved a ≥ 50% relative reduction in their overall SALT, score from baseline, which was a significant improvement compared to 8.6% for placebo ($p < 0.001$). The responders in the 8 mg twice-daily dose group were evenly distributed among patients with patchy alopecia areata and the more severe forms, alopecia totalis and alopecia universalis. The percentage of patients achieving the primary endpoint continued to increase up to Week 24, and regrowth of hair did not appear to plateau at Week 24. 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.

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

“We are very pleased that we were selected to present our initial safety and efficacy data with CTP-543 in patients with moderate to severe alopecia areata to the members of the Academy. We believe the results are highly encouraging and we are excited to share these data with the broader treatment community at the annual meeting,” stated James V. Cassella, Ph.D., Concert’s Chief Development Officer, who will make the oral presentation at AAD. “We look forward to advancing the development of CTP-543, as we continue our efforts to bring a new treatment to patients with this medical disease that currently has no approved medications.”

Details from the oral presentation, entitled “JAK Inhibitor CTP-543 Achieves Primary Endpoint in Phase 2a Trial in Alopecia Areata,” will be posted in the Scientific Presentations section of Concert’s website at the start of the oral presentation at the AAD on Saturday, March 2, 2019 at 1:20 p.m. ET.

The Phase 2 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. The primary outcome measure will utilize the Severity of Alopecia Tool (SALT) after 24 weeks of dosing. Patients were sequentially 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 fully enrolled and topline data for the overall study, including the 12mg cohort, is expected in the third quarter of 2019. Additional information about the trial (NCT03137381) is available on www.clinicaltrials.gov.

About CTP-543
CTP-543 was discovered by applying Concert's deuterium chemistry technology to modify ruxolitinib, a drug which 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.

About Alopecia Areata
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 FDA for the treatment of alopecia areata.

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 statements about our expectations for 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, availability of funding sufficient for our foreseeable and unforeseeable operating expenses and capital expenditure requirements 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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