Phase 2 Dose-Ranging Clinical Trial Results of CTP-543 in Patients with Alopecia Areata published in the Journal of the American Academy of Dermatology

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LEXINGTON, Mass.--(BUSINESS WIRE)--Jun. 7, 2022-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced the publication of safety and efficacy data from the randomized, double-blind, placebo-controlled dose-ranging Phase 2 clinical trial for CTP-543 in the *Journal of the American Academy of Dermatology (JAAD)*. CTP-543 is an investigational oral Janus Kinase (JAK) inhibitor being evaluated in ongoing clinical trials for the treatment of adults with moderate to severe alopecia areata, an autoimmune disease in which the immune system attacks hair follicles and results in hair loss. The publication reports clinically meaningful and statistically significant scalp hair regrowth after 24 weeks of treatment with CTP-543 in both the 8 mg twice-daily and 12 mg twice-daily dose groups in patients with alopecia areata.

“We have made so much progress in alopecia areata in so short a time, and it is utterly exciting to see JAK inhibitors advance as a potential treatment for alopecia areata,” said Brett King, M.D., Department of Dermatology, Yale University School of Medicine and lead author of the publication. “The therapeutic response in patients as early as 12 weeks in this trial is significant, and we hope that with continued development CTP-543 will become a treatment option for the many people suffering from this serious autoimmune disease.”

The publication highlights the establishment of two doses of CTP-543 with statistically significant hair regrowth and a generally well tolerated safety profile in patients with moderate to severe alopecia areata. Additionally, patient-reported outcomes of improvement following treatment with CTP-543 were statistically significant.

The robust clinical results observed in the trial supported the Breakthrough Therapy designation from the U.S. Food and Drug Administration (FDA) and the progression of CTP-543 into the THRIVE-AA pivotal Phase 3 clinical program. The Company recently reported positive topline data from the THRIVE-AA1 trial, and topline data from the THRIVE-AA2 trial is expected in the third quarter of 2022.

“We are very pleased that the results from this trial are published in this very prestigious journal and that its broad readership across the field of dermatology will see the first published results of CTP-543 in patients with alopecia areata,” said James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals and a co-author of the publication. “Today’s *JAAD* publication further emphasizes the growing body of literature showing the importance of the JAK mechanism in alopecia areata and the potential therapeutic utility of CTP-543 as a new treatment option for this important medical condition.”

CTP-543 Phase 2 Trial Results

The Phase 2 trial was a randomized, double-blind, 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 Severity of Alopecia Tool (SALT) after 24 weeks of dosing.

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 SALT score. 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. For patients in the 4 mg cohort, the relative reduction in their overall SALT score from baseline was not significantly different from placebo.

In the Phase 2 study, CTP-543 was generally well tolerated and safety results were consistent with the known safety profile of JAK inhibitors. There was one serious adverse event reported in the 12 mg dose group where the patient was able to complete the trial after a brief dose interruption.

A copy of the publication entitled, “Phase 2 randomized, dose-ranging trial of CTP-543, a selective Janus Kinase inhibitor, in moderate-to-severe alopecia areata” is available online on the *JAAD* website and supplemental materials are available on Mendeley.

About CTP-543 and Alopecia Areata

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

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 approximately 1.5 million 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 that is developing small molecule drugs that it discovered through the application of its DCE Platform® (deuterated chemical entity platform). Selective incorporation of deuterium into known molecules has the potential, on

a case-by-case basis, to provide better pharmacokinetic or metabolic properties, thereby enhancing their clinical safety, tolerability or efficacy. Concert's lead product candidate is in late-stage development for the treatment of alopecia areata, a serious autoimmune dermatological condition. Concert is also assessing a number of earlier-stage pipeline candidates. 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 regarding the development of CTP-543 and the timing of availability of clinical trial data, 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, timing and design of future clinical trials, the availability and timing of data from ongoing and future clinical trials and the results of such 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 Annual Report on Form 10-K 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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