

Concert Pharmaceuticals Announces Initiation of New Open Label Trial to Evaluate Once-Daily vs. Twice-Daily Dosing of CTP-543 in Patients with Alopecia Areata

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LEXINGTON, Mass.--(BUSINESS WIRE)--May 16, 2019-- [Concert Pharmaceuticals, Inc.](#) (NASDAQ: CNCE) today announced that it has initiated a second open label clinical trial to evaluate once-daily compared to twice-daily oral dosing of CTP-543 in patients with alopecia areata. Patients in the trial will be randomized to receive either 12 mg twice-daily or 24 mg once-daily of CTP-543 over a 24 week treatment period.

This new dose regimen trial is designed to complement the first open label trial initiated by Concert in March 2019, which is ongoing in patients randomized to receive either 8 mg twice-daily or 16 mg once-daily of CTP-543 over a 24 week treatment period. These two trials are designed to inform on the optimal dosing regimen for CTP-543 for future clinical trials.

"Our aim is to optimize the dosing regimen for CTP-543, so that we can best meet the needs of patients with this autoimmune disease for which there are currently no FDA-approved treatments," said James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. "We are pleased that our CTP-543 clinical efforts are helping to lead the way to develop a new treatment for alopecia areata."

The open label trial will enroll approximately 60 patients with moderate-to-severe alopecia areata in the United States and Canada. The trial will measure the relative change in Severity of Alopecia Tool (SALT) score between Week 24 and baseline. The trial is expected to be completed in the first half of 2020. All patients who complete 24 weeks of treatment will be eligible to enroll into an open label extension study of CTP-543. Additional information about the trial (NCT03941548) is available on www.clinicaltrials.gov.

About CTP-543 Interim Phase 2 Results

During the late-breaking clinical trials session at the American Academy of Dermatology (AAD) Annual Meeting on March 2, 2019, the Company presented interim results from an ongoing Phase 2 trial. At 24 weeks, patients treated with an 8 mg twice-daily dose of CTP-543 met the primary efficacy endpoint with a statistically significant difference compared to placebo ($p < 0.001$). The primary outcome measure was the proportion of patients with a $\geq 50\%$ relative reduction in their overall SALT score between Week 24 and baseline. As reported, CTP-543 was generally well tolerated and there were no serious adverse events reported. Dosing in an additional cohort receiving a 12 mg twice-daily dose of CTP-543 compared to placebo in the Phase 2 trial is currently ongoing. Additional information about the CTP-543 Phase 2 results is available in the [Scientific Presentations](#) section of Company's website.

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 time¹. 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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