Concert Pharmaceuticals Completes Enrollment of 12 mg Cohort in Phase 2a Trial of CTP-543 in Alopecia Areata

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LEXINGTON, Mass.--(BUSINESS WIRE)--Jan. 22, 2019-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced that it has completed patient enrollment of the final cohort evaluating 12 mg twice daily of CTP-543 in its Phase 2a trial for the treatment of moderate-to-severe alopecia areata. The trial previously enrolled patients to receive 4 mg and 8 mg twice daily compared to placebo. Data from the Phase 2a trial including the 12 mg cohort is expected in the third quarter of 2019.

“It is important early on in our development program to identify the safest and most effective doses of CTP-543 for patients with a chronic autoimmune disease like alopecia areata. The evaluation of the twice daily 12 mg dose provides a comprehensive exploration of the relevant dose range for CTP-543 and positions us well for later stage development of the compound,” stated James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals.

The Phase 2a 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. 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 primary outcome measure utilizes the severity of alopecia tool (SALT) after 24 weeks of dosing. Additional information about the trial is available on www.clinicaltrials.gov.

In November 2018, Concert reported positive interim topline results from the first two cohorts (4 mg and 8 mg twice daily) in its Phase 2a trial. The 8 mg twice daily cohort achieved the primary endpoint of ≥ 50% relative reduction in overall severity of alopecia tool (SALT) score from baseline at 24 weeks in 47% of patients compared to 8.6% of placebo patients ($p < 0.001$). The 4 mg twice daily CTP-543 cohort had numerically higher responses than placebo but was not statistically better than placebo. Regrowth of hair did not appear to plateau at Week 24. Treatment with CTP-543 was generally well tolerated. No serious adverse events were reported. Additional information about the CTP-543 Phase 2a results is available in the Scientific Presentations section of Company’s website.

About CTP-543 and Alopecia Areata

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

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 U.S. Food and Drug Administration (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 U.S. Food and Drug Administration’s (FDA) 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 currently 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 on the 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 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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