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Concert Pharmaceuticals Initiates CTP-543 Phase 2 Trial in Alopecia Areata

LEXINGTON, Mass.--(BUSINESS WIRE)-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced the initiation of a Phase 2a clinical trial evaluating CTP-543 for the treatment of moderate-to-severe alopecia areata, an autoimmune disorder in which the immune system attacks hair follicles, resulting in patchy or complete hair loss. CTP-543 is an orally-administered selective inhibitor of Janus kinases 1 and 2, known as JAK1 and JAK2, which are enzymes believed to be involved in this autoimmune disorder. The Phase 2a trial is designed to evaluate the safety and efficacy of CTP-543 after 12 months of dosing with the primary efficacy analysis at week 24. The Company expects to report topline data in the first quarter 2018.

"Alopecia areata is a devastating autoimmune disease that impacts half a million or more patients in the U.S. at any given time. There is a significant unmet medical need with no FDA-approved therapy," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "We intend to be at the forefront of advancing a new oral treatment for alopecia areata patients and look forward to assessing CTP-543's therapeutic efficacy in the Phase 2 trial."

"The treatment community, and patients suffering from this disease, have been eager for new treatments for alopecia areata. Though more work needs to be done to explore safety and efficacy, there is a growing and encouraging body of evidence supporting the Janus kinase mechanism in this indication," stated Julian Mackay-Wiggan, MD, MS., Associate Professor of Dermatology and Director of Clinical Research in Dermatology at Columbia University. "I believe this is a new era in which we are advancing treatments into clinical evaluation that target the underlying biology of the disease."

The Phase 2a trial is a double-blind, randomized, placebo-controlled, parallel dose trial to evaluate the safety and efficacy of CTP-543 in adult patients with moderate-to-severe alopecia areata. Approximately 100 patients will be randomized to receive one of four doses of CTP-543 (4, 8, 12 and 16 mg twice daily) or placebo. The primary outcome measure will utilize the severity of alopecia tool (SALT) after 24 weeks of dosing. The trial will include an additional 28 weeks of dosing where all patients enrolled in the study will receive CTP-543. Additional information about the trial will be available on www.clinicaltrials.gov.

Clinical Studies with CTP-543 and JAK Inhibitors in Alopecia Areata

In Phase 1 clinical studies to date with CTP-543 in healthy volunteers, the pharmacokinetic profile showed increased exposure with increasing doses of CTP-543. CTP-543 was well tolerated across all dose groups and there were no serious adverse events reported. The most commonly reported adverse event was headache. There were no withdrawals or dose modifications related to CTP-543. The average exposure observed with 16 mg of CTP-543 twice daily appeared to be comparable to published findings for the reported average exposure of 20 mg ruxolitinib twice daily. In an open-label clinical trial of 12 patients with alopecia areata, investigators at Columbia University reported that 20 mg of ruxolitinib administered orally twice-daily resulted in significant hair regrowth in 75% of individuals with moderate to severe alopecia areata[1].

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. Ruxolitinib has been used to treat alopecia areata in academic settings, including an investigator-sponsored clinical trial, and has been reported to promote hair growth in individuals with moderate-to-severe disease.

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[2]. 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 U.S. Food and Drug Administration (FDA) for the treatment of alopecia areata.

In 2015, the FDA selected alopecia areata as one of eight new disease areas that it will focus on in its Patient-Focused Drug Development Initiative (PFDDI) meetings during fiscal year 2016-2017. The goal of the PFDDI is to bring patient perspectives into an earlier stage of product development.

As part of Concert's activities with the treatment community, the Company is furthering the development of educational tools about alopecia areata. The Company has recently launched a website designed to provide educational content about research for new treatment options for those impacted with alopecia areata. To learn more, please visit, www.AlopeciaAreataResearch.com.

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 address unmet patient needs. The Company's approach starts with approved drugs in which deuterium substitution has the potential to enhance clinical safety, tolerability or efficacy. Concert has a broad pipeline of innovative medicines targeting pulmonary diseases, including cystic fibrosis, autoimmune and inflammatory diseases and central nervous systems (CNS) disorders. For more information please visit www.concertpharma.com.

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 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]JCI Insight. 2016;1(15):e89790. doi:10.1172/jci.insight.89790.

[2] 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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