CoNCERT Pharmaceuticals Inc.®
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FDA Grants Fast Track Designation to Concert Pharmaceuticals' CTP-543 for the Treatment of Alopecia Areata

CTP-543 is a Novel JAK Inhibitor in Phase 2 for Alopecia Areata as Potential First-in-Class Treatment

LEXINGTON, Mass.--(BUSINESS WIRE)-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for CTP-543, a novel, oral Janus kinase (JAK) inhibitor 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.

Fast Track designation is a process designed to facilitate the development and expedite the review of new therapies to treat serious conditions and address unmet medical needs. With Fast Track designation, early and frequent communications between the FDA and the sponsor are encouraged throughout the drug development and review process to help to ensure that questions are resolved quickly.

"Fast Track designation recognizes that alopecia areata is a serious disease in need of effective treatments. We look forward to collaborating with the FDA on the development of CTP-543," said James Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. "There are no FDA-approved treatments for alopecia areata and CTP-543 would represent a significant medical advance as a potential first-in-class treatment for moderate-to-severe disease."

Concert is conducting a multi-center, double-blind, randomized, placebo-controlled Phase 2a trial to evaluate the safety and efficacy of CTP-543 in adults with moderate-to-severe alopecia areata. For additional information on the ongoing CTP-543 Phase 2a trial, visit www.clinicaltrials.gov.

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 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 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. 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 FDA selected alopecia areata as one of eight new disease areas that it focused on under its Patient-Focused Drug Development Initiative (PFDDI) meetings in 2016-2017. The goal of the PFDDI is to bring patient perspectives into an earlier stage of product development. The meeting to discuss alopecia areata was held on Monday, September 11, 2017. Additional information is available online at:
https://www.fda.gov/ForIndustry/UserFees/PrescriptionDrugUserFee/ucm554443.htm

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 autoimmune and inflammatory 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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