

Concert Pharmaceuticals Announces Clinical Hold on CTP-543 Phase 2a Trial

-- FDA Requested Review of Recently Completed Non-Clinical Studies --

-- FDA Did Not Cite a Safety Concern --

LEXINGTON, Mass.--(BUSINESS WIRE)-- [Concert Pharmaceuticals, Inc.](#) (NASDAQ: CNCE) today announced that it has received notice from the U.S. Food and Drug Administration (FDA) that its CTP-543 Phase 2a clinical trial for alopecia areata has been placed on clinical hold. The FDA had previously informed the Company that it could initiate the Phase 2a clinical trial; however, subsequent to its initiation, but prior to dosing subjects, the FDA informed the Company that a review of certain recently completed non-clinical toxicology studies is required before proceeding. The FDA did not cite a safety concern; however, they intend to review these additional non-clinical data as support for the one-year dosing duration as planned in the Phase 2a trial.

At the request of the FDA, the Company intends to submit the requested non-clinical study reports promptly. By regulation, the FDA has 30 days from receipt of Concert's response to notify the company whether the clinical hold is lifted. Concert is working diligently with the FDA to resume enrollment in the Phase 2a trial as quickly as possible.

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.

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 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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