# Concert Pharmaceuticals Announces Plans for CTP-543 Phase 3 Trials in Alopecia Areata

April 1, 2020

LEXINGTON, Mass.--(BUSINESS WIRE)--Apr. 1, 2020-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced the details of its pivotal Phase 3 trials of CTP-543 in patients with moderate-to-severe alopecia areata, an autoimmune disorder that results in patchy or complete hair loss. Concert discussed key aspects of its planned Phase 3 trials in adult patients with the U.S. Food and Drug Administration (FDA) at a recently conducted End-of-Phase 2 meeting. The Phase 3 program is intended to support filing of a New Drug Application for CTP-543.

"The planned initiation of the Phase 3 program, combined with the positive results of the Phase 2 dose ranging trial, show the continued momentum for our development of CTP-543 for patients with alopecia areata," said James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. "Our Phase 3 clinical program is designed to confirm the previous findings observed with CTP-543 and advance our goal to bring an important new treatment option to people living with this life-altering autoimmune disease. Our intent is to initiate the Phase 3 program in the fourth quarter of 2020, pending COVID-19."

The planned Phase 3 program for CTP-543 will include two randomized, double-blind, placebo-controlled clinical trials in adults at sites in the U.S., Canada and Europe. The Phase 3 program will evaluate the Severity of Alopecia Tool (SALT) score after 24 weeks of dosing in patients with moderate-to-severe alopecia areata. Key aspects of the Phase 3 trials include:

- Patients age 18-65 years with ≥ 50% hair loss;
- Approximately 700 patients are expected to enroll in each of the two Phase 3 trials;
- Dosing: 8 mg twice-daily or 12 mg twice-daily of CTP-543 or placebo for 24 weeks; and
- Primary endpoint: Percent of patients achieving a SALT score ≤20 at Week 24.

The End-of-Phase 2 meeting with the FDA was requested as a result of positive data from the dose-ranging Phase 2 trial of CTP-543 in patients with moderate-to-severe alopecia areata. Patients treated with either 8 mg twice-daily or 12 mg twice-daily of CTP-543 met the primary efficacy endpoint with statistically significant differences ($p < 0.001$) relative to placebo in the percentage of patients achieving a ≥ 50% relative change from baseline at 24 weeks using SALT. The 8 mg twice-daily and 12 mg twice-daily groups were also significantly different from placebo in the number of patients achieving ≥ 75% and ≥ 90% relative change in SALT from baseline at 24 weeks. At Week 24, patients treated with 8 mg twice-daily and 12 mg twice-daily compared to placebo also rated significantly greater improvement in their alopecia areata on the Patient Global Impression of Improvement Scale. Treatment with CTP-543 was generally well tolerated, with one serious adverse event reported in the 12 mg dose group where the patient was able to complete the trial after a brief dose interruption.

**About CTP-543 and Alopecia Areata**

CTP-543 is an oral selective inhibitor of Janus kinases JAK1 and JAK2. The FDA has granted Fast Track designation for CTP-543 for the treatment of alopecia areata.

Alopecia areata is an autoimmune disease in which the immune system attacks hair follicles, resulting in partial or complete loss of hair on the scalp and body. Alopecia areata 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 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 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 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, among others, statements about our expectations regarding the design and timing of initiation of future clinical trials for CTP-543, and any other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "will," "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 and timing 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] 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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Justine E. Koenigsberg (Investors)
Concert Pharmaceuticals, Inc.
(781) 674-5284
ir@concertpharma.com

Kathryn Morris (media)
The Yates Network
(914) 204-6412
kathryn@theyatesnetwork.com

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