Concert Pharmaceuticals Presents Update on CTP-543 Long-Term Extension Study in Alopecia Areata During 2nd JAK Inhibitors Drug Development Summit

July 1, 2021

LEXINGTON, Mass.--(BUSINESS WIRE)--Jul. 1, 2021-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today will present an update from its ongoing open label, long-term extension study of the investigational medicine CTP-543 in patients with moderate to severe alopecia areata, an autoimmune disorder that results in patchy or complete hair loss. The data show that, relative to previous Phase 2 study results of CTP-543, hair regrowth assessed by the Severity of Alopecia Tool (SALT) was maintained or improved in the great majority of patients through one year of continuous dosing with 12 mg twice-daily of CTP-543. Approximately 57% of participants receiving 12 mg of CTP-543 twice-daily following 52 weeks of dosing achieved a clinically-meaningful SALT score of 20 or less. Dosing in the long-term extension study is ongoing, and patients completing the ongoing Phase 3 THRIVE-AA trials are eligible to enroll in the study. These data will be presented virtually during the virtual JAK Inhibitors Drug Development Summit.

“With its Breakthrough Therapy designation, we are excited about the prospect of CTP-543 for the treatment of alopecia areata as we advance it through its Phase 3 clinical program. Through the Phase 3 program and long-term extension study we are building a robust body of data to support our New Drug Application with the FDA, which we plan to file in early 2023,” said James V. Cassella, PhD, Concert’s Chief Development Officer, who made the oral presentation at the Summit. “We are committed to understanding how alopecia areata affects the whole person and are working diligently to advance CTP-543 as one of the first FDA-approved therapies for this serious autoimmune condition.”

“We believe Janus kinase inhibitors, or JAKs, are an important mechanism in the treatment of autoimmune disorders, including alopecia areata,” said Maryanne Senna, MD, principal investigator of the Hair Academic Innovative Research Unit at Massachusetts General Hospital. “Through clinical research, we are at a point where we better understand the utility of this class of medicines and believe for the first-time that new treatments could be on the horizon.”

CTP-543 has been generally well tolerated in the long-term extension study with only 4 of the 158 subjects entering the study discontinuing due to adverse events to date. Adverse events are consistent with those previously reported in the Phase 2 trials. Clinical labs for key hematology parameters appear stable across the long-term extension study relative to end of treatment in the Phase 2 trials.

Details from the oral presentation, entitled “Update on Long-Term Safety & Efficacy of CTP-543, an Oral JAK Inhibitor, for the Potential Treatment of Alopecia Areata” will be available at 11:55 a.m. ET in the Scientific Presentations section of Concert’s website in connection with the meeting presentation.

About CTP-543 and Alopecia Areata

CTP-543 is an oral selective inhibitor of Janus kinases JAK1 and JAK2. The U.S. Food and Drug Administration (FDA) has granted CTP-543 Breakthrough Therapy designation for the treatment of adult patients with moderate to severe alopecia areata and Fast Track designation 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 approximately 1 million 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 that is developing small molecule drugs that it discovered through the application of its DCE Platform® (deuterated chemical entity platform). Selective incorporation of deuterium into known molecules has the potential, on a case-by-case basis, to provide better pharmacokinetic or metabolic properties, thereby enhancing their clinical safety, tolerability or efficacy. Concert’s lead product candidate is in late-stage development for the treatment of alopecia areata, a serious autoimmune dermatological condition. Concert is also assessing a number of earlier-stage pipeline candidates. 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 development of CTP-543 and the timing of regulatory filings, 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, timing and design of future clinical trials, the availability and timing of data from ongoing and future clinical trials and the results of such 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] Benigno M. Clinical, Cosmetic and Investigational Dermatology 2020

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