Concert Pharmaceuticals Initiates THRIVE-AA1 Phase 3 Clinical Trial Evaluating CTP-543 for Alopecia Areata

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LEXINGTON, Mass.--(BUSINESS WIRE)--Nov. 3, 2020-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced it has initiated THRIVE-AA1, the first Phase 3 clinical trial to evaluate the efficacy and safety of CTP-543, an oral Janus kinase inhibitor, in adult patients with moderate to severe alopecia areata. The Company expects to report topline results from THRIVE-AA1 in 2022. A second Phase 3 trial, THRIVE-AA2, is expected to begin in the first half of 2021.

“Based on the results from our Phase 2 program, we believe CTP-543 has the potential to offer patients a best-in-class treatment for moderate to severe alopecia areata,” said James V. Cassella, Ph.D., Chief Development Officer, Concert Pharmaceuticals. “We are fully committed to advancing CTP-543 with its Breakthrough Therapy designation in order to make a meaningful difference in the lives of individuals impacted by alopecia areata.”

The initiation of THRIVE-AA1 follows an end-of-phase 2 meeting where Concert discussed key aspects of its Phase 3 program and registration strategy with the U.S. Food and Drug Administration (FDA). The end-of-phase 2 meeting with the FDA was held following positive results from a dose-ranging Phase 2 trial of CTP-543 in patients with moderate to severe alopecia areata. Under current timelines, the Company believes that positive results from two Phase 3 trials could serve as the basis for submitting a New Drug Application in early 2023 for CTP-543 for the treatment of moderate to severe alopecia areata in adult patients.

“There are millions of people around the world affected by and living with alopecia areata,” said Andy Bryant, Acting Chief Executive Officer of the National Alopecia Areata Foundation. “We're encouraged by CTP-543’s potential to be one of the first FDA-approved medicines to treat alopecia areata.”

About the THRIVE-AA1 Study

THRIVE-AA1 (NCT04518995) is a randomized, double-blind, placebo-controlled clinical trial in adult patients with moderate to severe alopecia areata at sites in the U.S., Canada and Europe evaluating the regrowth of scalp hair after 24 weeks of dosing using the Severity of Alopecia Tool (SALT). Key aspects of THRIVE-AA1 include:

- Patient characteristics: adults age 18-65 years with ≥ 50% hair loss are eligible for the study;
- Expected enrollment size: approximately 700 patients;
- 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.

For more information about the THRIVE-AA1 study, please visit: https://clinicaltrials.gov/ct2/show/NCT04518995.

About CTP-543 and Alopecia Areata

CTP-543 is an oral selective inhibitor of Janus kinases JAK1 and JAK2. The 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 up to 700,000 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 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 pipeline consists of clinical stage candidates targeting autoimmune and central nervous systems (CNS) disorders, and a number of preclinical compounds that it is currently assessing. 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 progress of clinical development of CTP-543, the timing of availability of clinical trial data, the timing of initiation of future clinical trials 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 and timing 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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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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