Concert Pharmaceuticals Initiates THRIVE-AA2 Phase 3 Clinical Trial Evaluating CTP-543 for Alopecia Areata

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LEXINGTON, Mass.--(BUSINESS WIRE)--May 27, 2021-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced it has initiated THRIVE-AA2, the second planned 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-AA2 in the second half of 2022. 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 to the U.S. Food and Drug Administration (FDA) in early 2023 for CTP-543 for the treatment of moderate to severe alopecia areata in adult patients.

“We expect the CTP-543 THRIVE-AA clinical program to provide the pivotal data needed to support our registration,” said James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. “The continued support of the alopecia areata community has been essential in progressing our program, and the initiation of this second Phase 3 trial brings us another step closer to seeking FDA-approval. We are hopeful that CTP-543 will be one of the first FDA-approved treatments for alopecia areata and will offer an important therapeutic option for this challenging autoimmune disease.”

“Alopecia areata can bring disruption and burden to daily life. But with the progress of clinical trials, there’s hope for patients,” said Thea Chassin, the Founder and Chief Executive Officer of Bald Girls Do Lunch Inc., an alopecia areata advocacy, education and support nonprofit. “We’re grateful for Concert’s research on behalf of the alopecia areata community. We applaud Concert’s goal to develop a potential new medication.”

About the THRIVE-AA2 Study

THRIVE-AA2 (NCT04797650) 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-AA2 include:

- Patient characteristics: adults age 18-65 years with ≥ 50% hair loss are eligible for the study;
- Expected enrollment size: approximately 440 patients;
- Dosing: 8 mg or 12 mg of CTP-543 or placebo twice-daily for 24 weeks; and
- Primary endpoint: Percent of patients achieving a SALT score ≤ 20 at Week 24.

For more information about the THRIVE-AA1 and THRIVE-AA2 studies, please visit www.clinicaltrials.gov.

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 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, the timing of availability of clinical trial data 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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