Concert Pharmaceuticals Reports Positive Topline Results for First CTP-543 Phase 3 Clinical Trial in Alopecia Areata

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THRIVE-AA1 Study Meets Primary Endpoint for Scalp Hair Regrowth and Key Secondary Endpoints at Both Doses

Statistically Significant Hair Regrowth Observed as Early as Eight Weeks

CTP-543 Has Potential to be Best-in-Class for the Treatment of Alopecia Areata

Topline Data from Second Phase 3 Trial, THRIVE-AA2, Expected Third Quarter of 2022

LEXINGTON, Mass.--(BUSINESS WIRE)--May 23, 2022-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced positive topline results from its recently completed Phase 3 clinical trial, THRIVE-AA1, evaluating its oral investigational medicine CTP-543 in adult patients with moderate to severe alopecia areata, an autoimmune disorder that results in patchy or complete scalp hair loss. The primary efficacy endpoint for THRIVE-AA1 was the percentage of patients achieving an absolute Severity of Alopecia Tool (SALT) score of 20 or less at Week 24 of treatment, which was met with statistical significance in both the 8 mg twice-daily and 12 mg twice-daily dose groups relative to placebo. Treatment with CTP-543 was generally well tolerated.

The key secondary endpoints were the percentage of responders on a Hair Satisfaction Patient Reported Outcome (PRO) scale at Week 24 and the percentage of patients achieving absolute SALT scores of 20 or less at each of Weeks 20, 16, 12 and 8. All key secondary endpoints were met with statistical significance in both dose groups.

“Today marks an important milestone in advancing new treatments for alopecia areata, and I’m so happy to see such positive results from the first Phase 3 trial with CTP-543,” said Brett King, M.D., Department of Dermatology, Yale University School of Medicine and clinical investigator of THRIVE-AA1. “There is a great need for treatments for this challenging disease, and the results from the THRIVE-AA1 trial suggest that CTP-543 may potentially provide an important therapy for treating alopecia areata.”

“With these compelling Phase 3 data, we believe that CTP-543 has the potential to be a best-in-class treatment for patients with alopecia areata, a disease that has long been ignored. We are extremely grateful to the patients and teams of clinical research professionals who participate in our trials,” said James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. “We’re working to change the treatment landscape and hope that CTP-543 will be one of the first FDA-approved treatment options for this serious disease.”

Patients enrolled in THRIVE-AA1 were required to have at least 50 percent scalp hair loss due to alopecia areata, as measured by SALT. A SALT score of 100 represents total scalp hair loss whereas a score of 0 represents no scalp hair loss. The average baseline SALT score across all patients was approximately 85.9 (corresponding to less than 15% average scalp hair coverage).

A statistically significant proportion of patients treated with either 8 mg twice-daily or 12 mg twice-daily of CTP-543 experienced greater scalp regrowth compared to placebo. The proportion of patients achieving a SALT score of 20 or less (meaning that 80 percent or more scalp hair coverage was achieved) was 41.5 percent in the 12 mg twice-daily dose group and 29.6 percent in the 8 mg twice-daily dose group, compared to 0.8 percent of patients in the placebo group, at the 24-week endpoint. The treatment difference for both dose groups of CTP-543 relative to placebo was statistically significant ($p<0.0001$).

The safety profile seen with CTP-543 in THRIVE-AA1 was consistent with previous studies. The most common (≥5%) side effects in any dose group were headache, acne, upper respiratory infection, increased creatine kinase levels, COVID-19 infection and nasopharyngitis. Upper respiratory infections were greater in the placebo group than in either of the CTP-543 dose groups. No pulmonary embolisms or deep vein thromboses were observed in the trial. One patient treated with the 8 mg twice-daily dose and one patient treated with the 12 mg twice-daily dose developed herpes zoster (shingles). Serious adverse events were reported in nine patients, with only one patient (in the 8 mg twice-daily dose group) having events (2) that were assessed as possibly related to treatment. Four patients who reported serious adverse events were in the placebo group.

Concert expects to submit the full results from this study for future scientific publication and presentation. These data, along with data from THRIVE-AA2, a second Phase 3 clinical trial, are intended to form the basis of a New Drug Application (NDA) planned to be submitted to the U.S. Food and Drug Administration (FDA) in the first half of 2023, assuming positive results from THRIVE-AA2. Topline data from THRIVE-AA2 are expected in the third quarter of 2022.

About THRIVE-AA1

THRIVE-AA1 (NCT04518995) is a randomized, double-blind, placebo-controlled clinical trial in 706 adult patients age 18-65 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 SALT score. Patients were randomized to receive either 8 mg twice-daily or 12 mg twice-daily of CTP-543 or placebo for 24 weeks. The primary endpoint is the percentage of patients achieving a SALT score ≤ 20 at 24 weeks. All patients who completed 24 weeks of treatment in THRIVE-AA1 had the opportunity to continue in a separate extension study to evaluate long-term safety and efficacy of CTP-543.

About CTP-543 and Alopecia Areata

CTP-543 is an investigational 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 approximately 1.5 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 potential for CTP-543 to be a best-in-class treatment for the treatment of alopecia areata, the timing of availability of clinical trial data and the timing of regulatory filings, including an NDA 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, 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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