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

August 1, 2022

THRIVE-AA2 Study Met Primary Endpoint for Scalp Hair Regrowth

Key Secondary Endpoint Met for Patient Reported Outcome on Hair Satisfaction

Key Secondary Endpoint Met for Hair Regrowth as Early as 12 Weeks

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

Company Completes Pivotal Program with Two Positive Phase 3 Trials and Expects to File New Drug Application in First Half of 2023

LEXINGTON, Mass.--(BUSINESS WIRE)--Aug. 1, 2022-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced positive topline results from its second Phase 3 clinical trial, THRIVE-AA2, 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-AA2 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.

“It is a new era of innovation for developing treatment options for patients with alopecia areata, many of whom often suffer physically and mentally with this challenging autoimmune disease,” said Maryanne Senna, MD, Director of the Lahey Hair Loss Clinic of Excellence and clinical investigator of THRIVE-AA2. “These results are very promising and suggest that CTP-543 has the potential to address important needs for people living with alopecia areata.”

“With the successful completion of our two THRIVE-AA Phase 3 trials, Concert is now moving rapidly to prepare our NDA for submission to the U.S. FDA in the first half of 2023. We are also continuing to advance our commercial planning as we position CTP-543 to potentially enter the marketplace for the large underserved population of patients with alopecia areata,” said James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. “Based on the results from our Phase 3 program, we believe CTP-543, if approved, could be a best-in-class treatment option for alopecia areata and have a distinct therapeutic profile with features that are important to patients, including the percentage of patients with clinically-meaningful hair regrowth, patient satisfaction levels, and hair regrowth rates.”

Dr. Cassella added, “The Concert team continues to be enthusiastic and committed to working to bring CTP-543 to market as soon as possible as a potential new treatment option for patients living with alopecia areata.”

Patients enrolled in THRIVE-AA2 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 87.9 (corresponding to approximately 12% 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 20 percent or less scalp hair loss) was 38.3 percent in the 12 mg twice-daily dose group and 33.0 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 key secondary endpoints were the percentage of responders on a Satisfaction of Hair Patient Reported Outcome (SPRO) 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. 47% of patients in the 8 mg twice-daily group and 52% of patients in the 12 mg twice-daily group reported being “satisfied” or “very satisfied,” as compared to 2% of patients in the placebo group. The treatment difference for both groups relative to placebo was statistically significant. SALT scores of 20 or less at Weeks 20, 16 and 12 were statistically significant in both dose groups.

The safety profile seen with CTP-543 in THRIVE-AA2 was consistent with previous studies. The most common (≥5%) side effects in any dose group were COVID-19 infection, nasopharyngitis, increased creatine kinase levels, acne and headache. No pulmonary embolisms or deep vein thromboses were observed in the trial. Two patients treated with the 8 mg twice-daily dose and two patients treated with the 12 mg twice-daily dose developed herpes zoster (shingles). Five serious adverse events were reported in five patients, with only one in the 8 mg twice-daily dose group that was assessed as possibly related to treatment.

Concert expects to submit the full results from this study for future scientific publication and presentation. These data, along with data from the first Phase 3 clinical trial, THRIVE-AA1, 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.

About THRIVE-AA2

THRIVE-AA2 (NCT04797650) is a randomized, double-blind, placebo-controlled clinical trial in 517 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-AA2 had the opportunity to continue in a separate extension study to evaluate long-term safety and efficacy of CTP-543. The THRIVE-AA pivotal program, consisting of two Phase 3 clinical trials that enrolled over 1,200 patients, is one of the most comprehensive development programs in alopecia areata.

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 limited treatment options available for 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 late-stage clinical 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 CTP-543 is being developed 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 and the planned timing for filing 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, including safety profiles, 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 the timing of the submission of an NDA, the availability of 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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Source: Concert Pharmaceuticals, Inc.