Concert Pharmaceuticals Announces Presentation of CTP-543 THRIVE-AA1 Phase 3 Data in Alopecia Areata During Late Breaking Session at EADV Congress

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35 Percent of Patients Treated With CTP-543 12 mg Twice-Daily Achieved At Least 90 Percent Scalp Hair Coverage (SALT Score ≤ 10) After 24 Weeks of Treatment

Newly-Reported Results Show Significant Improvement in Eyebrow and Eyelash Regrowth in Patients Treated With Both Doses of CTP-543

MILAN--(BUSINESS WIRE)--Sep. 10, 2022-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced the presentation of data from its Phase 3 clinical trial, THRIVE-AA1. The presentation highlights THRIVE-AA1 study results evaluating Concert's 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 THRIVE-AA1 data are being presented by Brett King, M.D., Department of Dermatology, Yale University School of Medicine and clinical investigator of THRIVE-AA1, during the 31st European Academy of Dermatology and Venereology (EADV) Congress Late Breaking News Session.

In the THRIVE-AA1 study, significant improvements in scalp hair regrowth compared to placebo were achieved at 24 weeks for patients taking 8 mg twice-daily and 12 mg twice-daily doses of CTP-543, as previously disclosed in the positive topline results reported by Concert earlier this year. Treatment with CTP-543 was generally well tolerated. The EADV presentation includes new data from the THRIVE-AA1 study showing the ability of CTP-543 to achieve more stringent criteria for hair regrowth than the study's primary endpoint of absolute Severity of Alopecia Tool (SALT) score of 20 or less (meaning 20 percent or less scalp hair loss) at Week 24. Specifically, 21 percent and 35 percent of the patients in the CTP-543 8 mg twice-daily and 12 mg twice-daily dose groups, respectively, achieved a SALT score of 10 or less at Week 24, compared to 0 percent of patients in the placebo group ($p<0.0001$). Also, the relative change in SALT score from baseline was significantly different for the 12 mg twice-daily dose group compared to placebo ($p<0.001$) as early as Week 4. In addition, new data are presented showing that patients in the THRIVE-AA1 study with loss of eyebrow or eyelash hair at baseline treated with CTP-543 had significant improvement compared to placebo over the 24-week treatment period ($p<0.001$).

"In the Phase 3 results to date, CTP-543 has hit the mark with the primary endpoint of SALT score of 20 or less, which is clinically meaningful scalp hair regrowth for patients with alopecia areata. It is notable that the majority of patients who achieve the primary endpoint achieve the more stringent endpoint of SALT score of 10 or less in the THRIVE-AA1 study," stated Dr. King. "These data are highly encouraging and support the potential of CTP-543 to regrow hair on the scalp, eyebrows and eyelashes in patients with alopecia areata, and in many cases with a rapid onset of effect."

"Based on the strength of the THRIVE-AA clinical program data, CTP-543 has the potential to be a best-in-class treatment for alopecia areata," stated James V. Cassella, Ph.D., Chief Development Officer at Concert Pharmaceuticals. "Alopecia areata is a serious autoimmune disorder that has a strong association with emotional and psychosocial impact on patients' lives. We hope that CTP-543 has the potential to bring broader benefit to alopecia areata patients and plan to file a New Drug Application for CTP-543 in the first half of 2023."

The Phase 3 data presented at EAVD include a comprehensive review of the THRIVE-AA1 results and are consistent with the topline data reported in May 2022 by Concert:

- The primary efficacy endpoint for THRIVE-AA1 was the percentage of patients achieving an absolute 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. 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 at Week 24 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. The treatment difference for both dose groups of CTP-543 relative to placebo was statistically significant ($p<0.0001$). In addition, 21 percent and 35 percent of the patients in the CTP-543 8 mg twice-daily and 12 mg twice-daily dose groups, respectively, achieved a SALT score of 10 or less at Week 24 compared to 0 percent of patients in the placebo group ($p<0.0001$).
- THRIVE-AA1 also met all the key secondary endpoints at both doses of CTP-543. 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. All key secondary endpoints were met with statistical significance in both dose groups.
- 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.

Details from the oral presentation, entitled "Top-Line Results from THRIVE-AA1: A Phase 3 Clinical Trial of CTP-543 (deuruxolitinib), an Oral JAK Inhibitor, in Adult Patients With Moderate to Severe Alopecia Areata," is available in the Scientific Presentations section of Concert's website.

About THRIVE-AA1

THRIVE-AA1 (NCT04518995) was 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 was the percentage of patients achieving a SALT score of 20 or less at 24 weeks.

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 in THRIVE-AA1 was approximately 85.9 (corresponding to less than 15% average scalp hair coverage).

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. More than 95% of eligible patients in THRIVE-AA1 elected to roll into the extension study.

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.

About Concert

Concert Pharmaceuticals is a late-stage clinical biopharmaceutical company that is developing CTP-543, a novel oral JAK1/2 inhibitor. Concert has successfully completed two Phase 3 trials with CTP-543 in adults with alopecia areata, a serious autoimmune dermatological condition. The Company is also evaluating the use of CTP-543 in other indications and 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 a New Drug Application (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.

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[1] Benigno M. Clinical, Cosmetic and Investigational Dermatology 2020

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