Concert Pharmaceuticals Announces Presentation of Deuruxolitinib THRIVE-AA1 Phase 3 Study Results in Alopecia Areata During World Congress for Hair Research

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New Analyses Presented on the Effect of Baseline Severity and Duration of Current Episode of Hair Loss on Scalp Hair Regrowth

MELBOURNE, Australia--(BUSINESS WIRE)--Nov. 21, 2022-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced the presentation of data from its THRIVE-AA1 Phase 3 clinical trial during the 12th World Congress for Hair Research. The presentation highlights THRIVE-AA1 study results evaluating Concert's investigational oral medicine deuruxolitinib (CTP-543) in adult patients with moderate to severe alopecia areata, an autoimmune disorder that results in patchy or complete scalp hair loss. The World Congress presentation includes new analyses from THRIVE-AA1 showing the effect of deuruxolitinib on regrowth of scalp hair based on disease severity and duration of current episode of hair loss. The data are being presented by Brett King, M.D., Department of Dermatology, Yale University School of Medicine and clinical investigator of THRIVE-AA1.

"These new data analyses are important to inform our treatment of patients with varying degrees of disease severity and duration of current episode of hair loss. For patients receiving deuruxolitinib in THRIVE-AA1, the data show meaningful and significant improvement in patients with the most severe disease and those with longer duration of current episode. The data show, however, that more patients succeed with treatment when they are treated before they lose all of their scalp hair and when they are treated earlier in an episode of severe loss" stated Dr. King.

In THRIVE-AA1, 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 deuruxolitinib, as previously disclosed in the positive topline results reported by Concert earlier this year. Treatment with deuruxolitinib was generally well tolerated.

The newly presented analyses on disease severity and duration of current episode of hair loss from THRIVE-AA1 include:

- For patients with an absolute Severity of Alopecia Tool (SALT) score less than 95 at baseline, 43% and 57% of the 8 mg twice-daily and 12 mg twice-daily deuruxolitinib dose groups, respectively, achieved a SALT score of 20 or less at Week 24, compared to 1% of patients in the placebo group ($p<0.0001$). The onset of effect was significant as early as Week 8 for both doses ($p<0.001$).
- For patients with a SALT score greater than or equal to 95 (representing complete or nearly complete scalp hair loss) at baseline, 20% and 30% of the 8 mg twice-daily and 12 mg twice-daily deuruxolitinib dose groups, respectively, achieved a SALT score of 20 or less at Week 24, compared to 0% of patients in the placebo group ($p<0.0001$). The onset of effect was significant as early as Week 12 for both doses ($p<0.01$).
- For patients whose duration of current episode of hair loss was less than 4 years, the proportion of patients achieving a SALT score of 20 or less by Week 24 was 33% and 48% in the deuruxolitinib 8 mg twice-daily and 12 mg twice-daily dose groups, respectively, compared to 1% of patients in the placebo group ($p<0.0001$).
- For patients whose duration of current episode of hair loss was greater than or equal to 4 years, the proportion of patients achieving a SALT score of 20 or less by Week 24 was 23% and 29% in the deuruxolitinib 8 mg twice-daily and 12 mg twice-daily dose groups, respectively, compared to 0% of patients in the placebo group ($p<0.0001$).

Deuruxolitinib was generally well-tolerated in THRIVE-AA1, consistent with its other Phase 2 and Phase 3 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 deuruxolitinib 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.

The data presented at the World Congress includes data recently presented at the 31st European Academy of Dermatology and Venereology (EADV) Congress late breaking news session. Details from the World Congress oral presentation, entitled "Results from a Phase 3 Trial, THRIVE-AA1, of the Oral JAK Inhibitor CTP-543 (Deuruxolitinib) 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 deuruxolitinib 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 deuruxolitinib. More than 95% of eligible patients in THRIVE-AA1 elected to roll into the extension study.

In THRIVE-AA1, 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 deuruxolitinib. Treatment with deuruxolitinib was generally well tolerated.

About Deuruxolitinib and Alopecia Areata

Deuruxolitinib (CTP-543) is an investigational oral selective inhibitor of Janus kinases JAK1 and JAK2. The Food and Drug Administration has granted deuruxolitinib 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 deuruxolitinib, a novel, deuterated, oral JAK1/2 inhibitor. Concert has successfully completed two Phase 3 trials with deuruxolitinib in adults with alopecia areata, a serious autoimmune dermatological disease. The Company is also evaluating the use of deuruxolitinib 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, Instagram or 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 deuruxolitinib, 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 a New Drug Application, 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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