Concert Pharmaceuticals Receives FDA Breakthrough Therapy Designation for CTP-543 for the Treatment of Alopecia Areata

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Designation Based on Phase 2 Data Demonstrating Significant Hair Regrowth in Patients with Alopecia Areata

LEXINGTON, Mass.--(BUSINESS WIRE)--Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced that the U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy Designation for the Company's oral Janus kinase inhibitor, CTP-543, for the treatment of adult patients with moderate-to-severe alopecia areata. There are currently no drugs approved by the FDA for the treatment of alopecia areata, an autoimmune disorder in which the immune system attacks hair follicles, resulting in patchy or complete hair loss.

FDA Breakthrough Therapy Designation is intended to expedite the development and review of medicines aimed at treating a serious or life-threatening disease where there is preliminary clinical evidence that the investigational therapy may offer substantial improvement over existing therapies.

"We are pleased that the FDA has recognized the therapeutic potential of CTP-543 as a new treatment in development for alopecia areata," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "Our goal is to bring this potential new therapy to patients as quickly as possible. We look forward to working closely with the FDA to expedite the development of CTP-543."

FDA Breakthrough Therapy Designation for CTP-543 is supported by positive data from a Phase 2 clinical trial in patients with moderate-to-severe alopecia areata. In September 2019, Concert reported that patients treated with either 8 mg twice-daily or 12 mg twice-daily of CTP-543 met the primary efficacy endpoint with statistically significant differences ($p < 0.001$) relative to placebo in the percentage of patients achieving a ≥ 50% relative change from baseline at 24 weeks as measured by Severity of Alopecia Tool (SALT). The 8 mg twice-daily and 12 mg twice-daily groups were also significantly different from placebo in the number of patients achieving a SALT score of 20 or less at 24 weeks, which is the primary efficacy endpoint that Concert intends to utilize in its pivotal registration studies. Treatment with CTP-543 was generally well tolerated, with one serious adverse event reported in the 12 mg dose group where the patient was able to complete the trial after a brief dose interruption.

As the next step in the CTP-543 clinical program, Concert intends to initiate its Phase 3 clinical program in the fourth quarter of 2020.

CTP-543 has also received Fast Track designation in the U.S. for the treatment of alopecia areata.

Additional information about the CTP-543 Phase 2 results is available in the Scientific Presentations section of the Company's website.

About the Phase 2 Trial Design of CTP-543 in Alopecia Areata

The Phase 2 trial was a double-blind, randomized, placebo-controlled, sequential dose trial to evaluate the safety and efficacy of CTP-543 in adult patients with moderate-to-severe alopecia areata. A total of 149 patients were randomized to receive one of three doses of CTP-543 (4 mg, 8 mg or 12 mg) or placebo, administered twice-daily. The primary outcome measure utilized the SALT score after 24 weeks of dosing. The average baseline SALT score across all patients was approximately 88%, where 0% is no scalp hair loss and 100% represents total scalp hair loss.

About CTP-543 and Alopecia Areata

CTP-543 is an oral selective inhibitor of Janus kinases JAK1 and JAK2. The FDA has granted Fast Track designation for CTP-543 for the treatment of alopecia areata. The Company intends to advance CTP-543 into Phase 3 evaluation in the fourth quarter of 2020.

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 approximately 700,000 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 focused on applying its DCE Platform® (deuterated chemical entity platform) to create novel medicines designed to treat serious diseases and address unmet patient needs. The Company's approach starts with previously studied compounds, including approved drugs, in which deuterium substitution has the potential to enhance clinical safety, tolerability or efficacy. Concert's pipeline of innovative medicines targets autoimmune diseases and central nervous systems (CNS) disorders. 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 on the progress of clinical development of 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 and timing of future clinical 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.

Contacts

Justine Koenigsberg (investors)
Concert Pharmaceuticals, Inc.
(781) 674-5284
ir@concertpharma.com

Kathryn Morris (media)
The Yates Network
(914) 204-6412
kathryn@theyatesnetwork.com