Concert Pharmaceuticals Presents CTP-543 Data from Long-Term Extension Study in Alopecia Areata During Late-Breaking Session at EADV Congress

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LEXINGTON, Mass.--(BUSINESS WIRE)--Oct. 29, 2020-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today presented initial data from the ongoing long-term, open-label extension study of the investigational medicine CTP-543 in patients with moderate to severe alopecia areata, an autoimmune disorder that results in patchy or complete hair loss. The data from this long-term extension study show that, relative to previous Phase 2 study results of CTP-543, hair regrowth assessed by the Severity of Alopecia Tool (SALT) was maintained or improved in the vast majority of patients to date. These data were presented during the 29th European Academy of Dermatology and Venereology (EADV) Virtual Congress.

"We are pleased to offer patients participating in our trials the ability to continue to receive CTP-543 as part of our long-term assessment of the candidate," stated James V. Cassella, Ph.D., Concert's Chief Development Officer, who made the oral presentation at EADV. "In addition to providing participants the opportunity to continue treatment beyond the defined 24-week clinical trial period, we are also collecting important long-term safety data and evidence of CTP-543's effect on hair regrowth to support future registration. We are excited about our growing momentum as we advance CTP-543 with its Breakthrough Therapy designation and expect to begin our first Phase 3 trial of CTP-543 in patients with moderate to severe alopecia areata shortly."

More than 90% of eligible patients have enrolled in the CTP-543 long-term extension study. To date, approximately 130 patients have been dosed with CTP-543 for more than 1 year, and over 50 patients have been dosed for more than 1.5 years. Treatment with CTP-543 shows continued maintenance of hair regrowth across the long-term extension study. Patients continuing to receive 12 mg twice daily generally show consistent hair regrowth, with an average absolute SALT score of approximately 40 or less at entry and throughout the long-term extension study. Patients receiving 8 mg twice daily in the previously-completed Phase 2 trial who escalated to 12 mg in the long-term extension study generally show continued improvement of SALT scores at the higher dose.

CTP-543 has been generally well tolerated in the long-term extension study, with clinical labs appearing stable and the adverse events profile being generally consistent with that previously reported in the Phase 2 trials, the majority of which were reported as mild to moderate. The presentation reported that five serious adverse events have occurred in the long-term extension study, two of which were possibly related to CTP-543 and the other three of which were not or unlikely related to CTP-543.

Details from the oral presentation, entitled "Initial Results from a Long-Term, Open-Label Extension Study with CTP-543, an Oral Janus Kinase Inhibitor, in Patients with Moderate to Severe Alopecia Areata" is available in the Scientific Presentations section of Concert's website.

About CTP-543 and Alopecia Areata

CTP-543 is an oral selective inhibitor of Janus kinases JAK1 and JAK2. The U.S. Food and Drug Administration (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 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 the timing of initiation of future clinical trials, 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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