December 14, 2016

Concert Pharmaceuticals Announces CTP-543 Positive Top-Line Phase 1 Results

CTP-543 to Advance into Phase 2 in First Quarter of 2017

LEXINGTON, Mass.--(BUSINESS WIRE)-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced the Phase 1 single and multiple ascending dose trial evaluating CTP-543 in healthy volunteers has been completed. Concert is developing CTP-543 for the treatment of alopecia areata, an autoimmune disorder in which the immune system attacks hair follicles, resulting in patchy or complete hair loss. CTP-543 is a deuterium-modified analog of ruxolitinib, a Janus Kinase (JAK) inhibitor that is commercially available under the brand name Jakafi® for the treatment of myelofibrosis and for polycythemia vera. The Phase 1 program was designed to determine the safety, tolerability and pharmacokinetics of CTP-543 as well as determine doses for the planned Phase 2a clinical trial. Given the overall exposure and safety parameters observed, the Company has selected four doses (4, 8, 12 and 16 mg BID) to explore in its Phase 2a trial. The Phase 2a clinical trial is expected to begin in the first quarter of 2017.

"We are pleased with the pharmacokinetic profile emerging in our Phase 1 development with CTP-543," said James Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. "There has been converging evidence that JAK inhibition is important in promoting hair growth in individuals with alopecia areata, representing a major advance in developing a new treatment for this condition. We look forward to bringing CTP-543 into its first efficacy study next year and believe we have the potential to be the first to market with an FDA-approved oral treatment."

The single and multiple ascending dose trial enrolled a total of 77 healthy volunteers. The pharmacokinetic measurements showed increased exposure with increasing doses. The half-life of CTP-543 was approximately 3.3 hours, similar to that reported for non-deuterated ruxolitinib[1]. CTP-543 was well-tolerated across all dose groups and there were no serious adverse events reported in subjects who received CTP-543.

The safety and exposure observed with 16 mg of CTP-543 twice daily appeared comparable to the reported exposure of 20 mg ruxolitinib twice daily. Published findings from an open-label clinical trial of 12 patients with alopecia areata conducted by investigators at Columbia University demonstrated that 20 mg of ruxolitinib administered twice daily resulted in significant hair regrowth in 75% of patients with moderate to severe alopecia areata[2].

The Company intends to present the Phase 1 findings at a medical conference in 2017.

The Company's planned Phase 2a trial, which will enroll approximately 100 patients with moderate to severe alopecia areata, is a dose-ranging trial with four active arms and a placebo comparator. The primary outcome measure of the Phase 2a trial will utilize the severity of alopecia tool (SALT) after 24 weeks of dosing. The trial will include an extension arm where all patients enrolled in the study will receive CTP-543 for an additional 28 weeks of dosing. The trial is expected to commence in the first quarter of next year and topline primary outcome data is expected by the end of 2017.

About CTP-543 and About Alopecia Areata

CTP-543 was discovered by applying Concert's deuterium chemistry technology to modify ruxolitinib, which is commercially available under the name Jakafi® in the United States for the treatment of myelofibrosis and polycythemia vera. Ruxolitinib has been used to treat alopecia areata in academic settings, including an investigator-sponsored clinical trial, and has been shown to promote hair growth in individuals with moderate to severe disease.

Alopecia areata is an autoimmune disease that results in partial or complete loss of hair on the scalp and body that may affect up to 650,000 Americans at any given time[3]. 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 U.S. Food and Drug Administration (FDA) for the treatment of alopecia areata.

In 2015, the FDA selected alopecia areata as one of eight new disease areas that it will focus on under its Patient-Focused Drug Development Initiative (PFDDI) meeting during fiscal year 2016-2017. The goal of the PFDDI is to bring patient perspectives into an earlier stage of product development.

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 address unmet patient needs. The Company's approach starts with approved drugs in which deuterium substitution has the potential to enhance clinical safety, tolerability or efficacy. Concert has a broad pipeline of innovative medicines targeting pulmonary diseases, including cystic fibrosis, central nervous systems (CNS) disorders, as well as autoimmune and inflammatory diseases. For more information please visit www.concertpharma.com.

Cautionary Note on Forward Looking Statements

Any statements in this press release about our future expectations, plans and prospects, including statements about our expectations on the clinical development of CTP-543, and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "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 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.

References:

[1] Shi et al, *J Clin Pharmacol*, 2011.

[2] *JCI Insight*. 2016;1(15):e89790. doi:10.1172/jci.insight.89790.

[3] Fricke M. Epidemiology and Burden of alopecia areata: a systemic review. Clinical, Cosmetic and Investigational Dermatology. 2015; Vol 8. 397-403.

View source version on businesswire.com: http://www.businesswire.com/news/home/20161214005181/en/

Concert Pharmaceuticals, Inc.
Justine Koenigsberg (investors), 781-674-5284
ir@concertpharma.com
or
The Yates Network
Kathryn Morris (media), 845-635-9828

Source: Concert Pharmaceuticals, Inc.

News Provided by Acquire Media