Concert Pharmaceuticals Completes Enrollment in CTP-692 Phase 2 Trial in Patients with Schizophrenia

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CTP-692 Phase 2 Topline Results Expected First Quarter of 2021

LEXINGTON, Mass.--(BUSINESS WIRE)--Sep. 29, 2020-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced that it has completed patient enrollment of its Phase 2 clinical trial to evaluate CTP-692 as an adjunctive treatment in patients with schizophrenia. Topline data from the trial are expected in the first quarter of 2021.

"CTP-692 offers a potential new mechanism to treat schizophrenia that, when combined with existing antipsychotic medications, may more broadly address disease symptoms than is currently possible. We designed the Phase 2 study to assess CTP-692's potential to improve on the primary symptom domains in schizophrenia, including positive and negative symptoms and cognitive function, when added to existing antipsychotic treatments," said James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. "Maintaining the integrity of our trial has been a top priority, and we're grateful to the study sites and patients for enabling us to complete enrollment and keep the study progressing forward despite the impact of COVID-19."

The double-blind, randomized, placebo-controlled Phase 2 trial is designed to evaluate the safety and efficacy of CTP-692 as an adjunctive treatment in adult patients with schizophrenia. A total of 325 patients already on a stable course of an antipsychotic medication were randomized to receive 1, 2, or 4-gram doses of CTP-692 or placebo once-daily. The primary outcome measure is the change in the Positive and Negative Syndrome Scale (PANSS) total score at 12 weeks compared to baseline. Additional information about the trial (NCT04158687) is available on www.clinicaltrials.gov.

About CTP-692 and Schizophrenia

CTP-692 is an investigational deuterium-modified form of D-serine that offers an entirely new mechanism of action to potentially treat schizophrenia and more broadly address its symptoms than has been previously possible. The underlying rationale of the current antipsychotic therapies for schizophrenia is that excessive dopaminergic neurotransmission and dysfunctional D2 receptor signaling are believed to play key pathophysiological roles in the disease, and consequently all approved typical and atypical antipsychotics possess some level of D2 antagonist activity. However, deficient glutamatergic neurotransmission mediated by the NMDA receptor is believed to be another underlying cause of schizophrenia. It has been reported that individuals with schizophrenia have low plasma and cerebrospinal fluid levels of D-serine, a key molecule that activates NMDA receptors in areas of the brain that are widely believed to play key roles in schizophrenia. CTP-692 administration may help enhance NMDA neurotransmission, potentially more safely than using unmodified D-serine. CTP-692 is being initially developed as an adjunctive therapy with the potential to improve positive and negative symptoms as well as cognitive function in patients with schizophrenia.

Schizophrenia is a chronic and devastating neuropsychiatric disorder that is ranked as a leading cause of disability worldwide. The disease afflicts nearly 1% of the world's population, affecting both men and women, and striking all ethnic and socioeconomic groups with a similar level of prevalence. The illness is characterized by multiple symptoms that are categorized into three main clusters known as positive symptoms (hallucinations, delusional behaviors and thought disorder), negative symptoms (social withdrawal, flattened affect and poverty of speech), and cognitive dysfunction (diminished capacity for attention, working memory and executive function). Currently available antipsychotic drugs offer some benefit for positive symptoms but are frequently associated with neurologic and metabolic adverse effects and are limited in their capacity to treat negative symptoms and cognitive dysfunction, which are related to poor functional outcomes.

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 currently 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 regarding the progress of clinical development of CTP-692 and the timing of availability of clinical trial data, 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, 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.

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