Concert Pharmaceuticals Initiates CTP-692 Phase 2 Trial in Schizophrenia

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CTP-692 Offers Potentially New Approach as an Adjunctive Treatment to Improve Overall Symptoms in Patients with Schizophrenia

LEXINGTON, Mass.--(BUSINESS WIRE)--Dec. 2, 2019-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced the initiation of a Phase 2 clinical trial evaluating CTP-692 as an adjunctive treatment for schizophrenia. CTP-692 is a deuterated form of D-serine, an endogenous amino acid that is a co-agonist of the NMDA receptor. NMDA is believed to play a key role in schizophrenia. The Phase 2 trial is designed to evaluate the safety and efficacy of three different doses of CTP-692, compared with placebo, in patients with schizophrenia who are stable on an antipsychotic medication. The Company expects to report topline Phase 2 data by year end 2020.

The Company recently hosted a webcast event featuring a discussion with Dr. Joseph T. Coyle, Director, Laboratory for Psychiatric and Molecular Neuroscience at McLean Hospital and Eben S. Draper Chair of Psychiatry and Neuroscience at Harvard Medical School, where Dr. Coyle reviewed the current understanding of the causes of schizophrenia. During the event Dr. Coyle commented: “Recent genetic and brain imaging results provide compelling evidence of impaired function of a brain receptor central to neuroplasticity, learning and memory. A critical co-agonist at this receptor is D-serine. Patients with schizophrenia have deficient levels of D-serine in the plasma, CSF and brain, and CTP-692 could help to restore those levels and improve schizophrenia symptoms that are not adequately addressed with current treatments.”

“We are pleased with the rapid progress we have made to advance CTP-692 into this Phase 2 study in schizophrenia patients, with the potential to be the first approved adjunctive treatment for schizophrenia. We believe that CTP-692 has potential to realize the beneficial effects of D-serine to improve schizophrenia symptoms, including positive, negative and cognitive symptoms, without the risk of renal safety concerns known for D-serine,” said James V. Cassella, Ph.D., Chief Development Officer of Concert Pharmaceuticals. “CTP-692 works through a different mechanism of action than existing antipsychotic treatments and therefore offers the potential for broader symptom improvement when added to existing antipsychotic treatments.”

The Phase 2 trial is a double-blind, randomized, placebo-controlled trial to evaluate the safety and efficacy of CTP-692 in adult patients with schizophrenia. Approximately 300 patients who are stable on an antipsychotic medication will be randomized to receive 1, 2 or 4 grams 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 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 treat schizophrenia, potentially more broadly addressing its symptoms than has been previously possible. The underlying basis of the current antipsychotic therapies for schizophrenia is that excessive dopaminergic neurotransmission and dysfunctional D2 receptor signaling play key pathophysiological roles in the disease, and consequently all approved typical and atypical antipsychotics possess some level of D2 antagonist activity. However, it has been reported that individuals with schizophrenia have low 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 address this D-serine deficiency. 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.

KOL Webcast Event

A replay of the November 14, 2019 webcast featuring Dr. Joseph T. Coyle of McLean Hospital and Harvard Medical School will be available on Concert’s website at www.concertpharma.com until February 2020.

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 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 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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