Concert Pharmaceuticals Announces Advancement of Novel Drug Candidate in Schizophrenia

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CTP-692 Poised to Enter Clinical Development in 2018 as an Adjunctive Treatment of Schizophrenia

Conference Call Scheduled Tomorrow at 8:30 a.m. ET for Company Update and 2017 Financial Results

LEXINGTON, Mass.--(BUSINESS WIRE)--Feb. 28, 2018-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today announced the next development candidate in its product pipeline with the selection of CTP-692, a novel drug for adjunctive treatment of schizophrenia, a devastating, chronic illness with significant unmet need. CTP-692 is a deuterated form of D-serine, an endogenous, required co-agonist of the N-methyl-D-aspartate (NMDA) receptor. NMDA receptor hypofunction is believed to contribute to the pathophysiology of schizophrenia and enhancement of D-serine levels is believed to benefit individuals with schizophrenia. CTP-692 is being developed as a potential adjunctive therapy to antipsychotic medicines with the potential to improve positive and negative symptoms as well as cognitive function in these patients. The Company intends to complete preclinical evaluation and advance CTP-692 into clinical development by year-end 2018.

“We see neuropsychiatry as an area in which our team is well-positioned to broaden and expand our pipeline and apply our deuterium chemistry technology to create innovative medicines with differentiated therapeutic properties,” said Roger Tung, Ph.D., President and CEO of Concert Pharmaceuticals. “Our newest development candidate, CTP-692, represents an exciting opportunity to improve upon the existing standard-of-care with the potential to make a meaningful difference for patients with schizophrenia.”

Despite its therapeutic potential as an adjunctive antipsychotic medication, the development of D-serine has been limited by safety concerns. D-serine has been shown to cause nephrotoxicity in preclinical testing. In addition, laboratory findings suggesting a possible renal safety signal were observed in some patients who received D-serine in clinical studies.

CTP-692 has the potential to improve the safety profile of D-serine. In preclinical evaluation, Concert found that selective deuterium modification increased D-serine exposure and substantially reduced evidence of renal impairment. As a result, the Company believes that it can explore a wider exposure range to achieve optimal therapeutic levels in the clinic with a much lower risk of renal toxicity. These results support the further advancement of CTP-692.

About CTP-692 and Schizophrenia

CTP-692 is a deuterium-modified analog of endogenous D-serine. Based on documented effects of D-serine, the Company believes that CTP-692 has the potential to restore NMDA receptor activity in key areas of the brain and improve clinical outcomes in patients with schizophrenia. CTP-692 is expected to have similar pharmacology to D-serine with the potential for an improved safety profile and improved clinical outcomes in the treatment of schizophrenia. CTP-692 will be developed as an adjunctive therapy administered in addition to standard antipsychotic medicines to improve both positive and negative symptoms as well as cognitive function in patients with schizophrenia.

There is an extensive body of evidence supporting NMDA receptor hypofunction as a key underlying mechanism of schizophrenia. The NMDA receptor comprises two binding domains and, in addition to requiring glutamate binding, activation with a co-agonist such as D-serine or glycine is necessary for NMDA receptor activation. D-serine is the most important endogenous co-agonist for synaptic transmission in the human central nervous system. It has been postulated for some time that administration of NMDA co-agonists could benefit patients with schizophrenia since there is evidence that plasma and CSF levels of endogenous D-serine are reduced 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 equally, 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). The underlying basis of the current antipsychotic therapy is that excessive dopaminergic neurotransmission and dysfunctional D2 receptor signaling play key pathophysiological roles in the disease, and consequently all typical and atypical antipsychotics in clinical practice possess some level of D2 antagonist activity. Currently available antipsychotic drugs exhibit efficacy for positive symptoms, but are limited in their capacity to treat negative symptoms and cognitive dysfunction which are related to poor functional outcomes for these patients.

Conference Call and Webcast

The Company will host a conference call and webcast tomorrow, March 1, 2018 at 8:30 a.m. ET to provide an update on the company and discuss fourth quarter 2017 financial results. To access the conference call, please dial (855) 354-1855 (U.S. and Canada) or (484) 365-2865 (International) five minutes prior to the start time.

A live webcast of Concert’s presentation may be accessed in the Investors section of the Company’s website at www.concertpharma.com. Please log on to the Concert website approximately 15 minutes prior to the scheduled webcast to ensure adequate time for any software downloads that may be required. A replay of the webcast will be available on Concert’s website for three months.

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 starts with previously studied compounds, including 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 autoimmune and inflammatory 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 statements about clinical development of CTP-692 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, 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 Annual Report on Form 10-K 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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