Concert Pharmaceuticals Reports Third Quarter 2019 Financial Results and Provides Company Update

November 7, 2019

Conference Call Scheduled Today at 8:30 a.m. ET

LEXINGTON, Mass.--(BUSINESS WIRE)--Nov. 7, 2019-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today reported financial results for the third quarter of 2019.

"Over the past several months, we have presented important clinical advances for both of our proprietary candidates – CTP-543 for alopecia areata and CTP-692 for schizophrenia – which support their continued development, and have been broadly shared with the clinical community," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "We believe that our CTP-543 Phase 2 data set a new bar for clinical efficacy in the treatment of alopecia areata. Additionally, we are pleased that CTP-692 demonstrated favorable clinical properties including an excellent safety profile in its Phase 1 studies, and we look forward to advancing it into its first efficacy trial later this year."

Recent Business Highlights and Upcoming Milestones

CTP-543: an investigational treatment for moderate-to-severe alopecia areata

- ***CTP-543 Phase 2 Data Supports Advancement into Phase 3.*** In September 2019, Concert reported that patients treated with either 8 mg twice-daily or 12 mg twice-daily of CTP-543 met the primary efficacy endpoint with statistically significant differences ($p < 0.001$) relative to placebo in the percentage of patients achieving a ≥ 50% relative change from baseline at 24 weeks as measured by Severity of Alopecia Tool (SALT). The 8 mg twice-daily and 12 mg twice-daily groups were also significantly different from placebo in the number of patients achieving ≥ 75% and ≥ 90% relative change in SALT from baseline at 24 weeks. Treatment with CTP-543 was generally well tolerated, with one serious adverse event reported in the 12 mg dose group where the patient was able to complete the trial after a brief dose interruption. Based on the totality of the data, the Company intends to advance CTP-543 into Phase 3 development in 2020.
- ***CTP-543 Phase 2 Data Presented in Late Breaking News Session at EADV 2019.*** Dose-ranging Phase 2 data were presented in the late breaking news session at the European Academy of Dermatology and Venereology (EADV) Annual Congress on October 12, 2019, in Madrid, Spain. The presentation highlighted the Phase 2 results in which CTP-543 met the primary efficacy endpoint for the 8 mg twice-daily and 12 mg twice-daily doses with greater hair regrowth on the scalp compared to placebo. The presentation also described patient data showing significantly greater self-reported improvement in their alopecia areata on the Patient Global Impression of Improvement Scale at Week 24 for the 8 mg and 12 mg twice-daily doses compared to placebo.
- ***CTP-543 Open Label Trial Evaluating 12 mg Twice-Daily vs. 24 mg Once-Daily Fully Enrolled.*** In September 2019, the Company completed patient enrollment of an open label clinical trial to evaluate 24 mg once-daily compared to 12 mg twice-daily oral dosing of CTP-543 in adult patients with alopecia areata. Topline data from this dose regimen trial is expected in the first half of 2020. An open label clinical trial assessing 16 mg once-daily compared to 8 mg twice-daily oral dosing of CTP-543 completed enrollment in June 2019 with topline results expected in the fourth quarter of 2019.

CTP-692: an investigational adjunctive treatment for schizophrenia

- ***CTP-692 Webcast with Key Opinion Leader***. On November 14, 2019, the Company will host a webcast for investors to highlight the CTP-692 program. The event will include a presentation on mechanism and need for new treatments in schizophrenia by Dr. Joseph Coyle, Director, Laboratory for Psychiatric and Molecular Neuroscience at McLean Hospital and Harvard Medical School. The live webcast and replay of the event, with slides, can be accessed on the Events & Presentations page of the Investors section of Concert's corporate website.
- ***CTP-692 Phase 1 Results Presented at ECNP Annual Congress.*** The Company presented its CTP-692 Phase 1 single- and multiple-ascending dose clinical results during a poster session at the European College of Neuropsychopharmacology Congress on September 10, 2019, in Copenhagen, Denmark. The safety assessments in the single- and multiple-ascending dose Phase 1 trials in healthy volunteers showed that CTP-692 was generally well tolerated over the dose ranges tested, which included the doses to be evaluated in Phase 2 testing. Importantly, key blood and urine markers of kidney function did not indicate any signs of renal impairment.
- ***CTP-692 Phase 2 Trial Initiation Planned in Fourth Quarter of 2019***. The Company intends to advance CTP-692 into Phase 2 testing in the fourth quarter of 2019. CTP-692 will be initially developed as an adjunctive therapy administered in addition to standard antipsychotic medicines with the potential to improve positive and negative symptoms as well as cognitive function in patients with schizophrenia.

AVP-786 for Neurological Disorders

- ***Avanir and Otsuka Report AVP-786 Phase 3 Topline Results.*** In September 2019, Avanir Pharmaceuticals and Otsuka Pharmaceuticals announced that a recently completed Phase 3 trial evaluating the efficacy, safety and tolerability of AVP-786 for the treatment of moderate-to-severe agitation in patients with Alzheimer's dementia did not meet its primary and key secondary endpoints. Avanir and Otsuka have stated that they will analyze the full set of data from the first two studies of the AVP-786 Phase 3 clinical development program and explore the best path forward.

Corporate Developments

- ***Jeffrey Munsie Joins Concert as Chief Legal Officer.*** In September 2019, Concert named Jeffrey A. Munsie to the position of Chief Legal Officer. In this role, Mr. Munsie will have overall responsibility for key general corporate legal functions, including the Company's public reporting requirements, governance and intellectual property matters, and will serve as corporate secretary.

Third Quarter 2019 Financial Results

- ***Cash and Investment Position.*** Cash, cash equivalents and investments as of September 30, 2019 totaled $121.5 million. The Company expects its cash, cash equivalents and investments to be sufficient to fund the Company through 2020 under its current operating plan.
- ***R&D Expenses.*** Research and development expenses were $13.5 million for the quarter ended September 30, 2019, compared to $11.0 million for the same period in 2018. The increase in research and development expenses relates primarily to the clinical development of CTP-543, as well as increased expenses associated with the manufacturing of CTP-692 to support ongoing clinical development.
- ***G&A Expenses.*** General and administrative expenses were $4.7 million for the quarter ended September 30, 2019, compared to $6.3 million for the same period in 2018. Decreases in legal and employee-related expenses were partially offset by an increase in professional fees.
- ***Net Loss.*** For the quarter ended September 30, 2019, net loss applicable to common stockholders was $17.2 million, or $0.72 per share, compared with a net loss applicable to common stockholders of $17.4 million, or $0.74 per share, for the quarter ended September 30, 2018.

Conference Call and Webcast

The Company will host a conference call and webcast today at 8:30 a.m. ET to provide an update on the company and discuss third quarter 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.

Concert Pharmaceuticals, Inc.

Condensed Consolidated Statements of Operations

(in thousands, except per share amounts)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Revenue:				
License and research and development revenue	$ 10	$ 11	$ 1,064	$ 10,492
Operating expenses:				
Research and development	13,511	11,031	43,797	28,549

General and administrative	4,742	6,320	15,329	17,464
Total operating expenses	18,253	17,351	59,126	46,013
Loss from operations	(18,243)	(17,340)	(58,062)	(35,521)
Investment income	724	703	2,474	2,003
Unrealized gain (loss) on marketable equity securities	334	(732)	(2,091)	(1,359)
Loss before tax provision	(17,185)	(17,369)	(57,679)	(34,877)
Provision for income taxes	—	18	—	298
Net loss	$ (17,185)	$ (17,387)	$ (57,679)	$ (35,175)
Net loss per share applicable to common stockholders — basic and diluted	$ (0.72)	$ (0.74)	$ (2.43)	$ (1.51)
Weighted-average number of common shares used in net loss per share applicable to common stockholders— basic and diluted	23,807	23,421	23,703	23,349

Concert Pharmaceuticals, Inc.

Summary Balance Sheet Data

(in thousands)

	September 30, 2019	**December 31, 2018**
Cash and cash equivalents	$ 33,384	$ 17,770
Investments, available for sale	88,165	135,544
Working capital	117,362	171,400
Total assets	151,957	192,547
Deferred revenue	10,533	10,533
Total stockholders' equity	119,263	167,740

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 CTP-692, the timing of availability of clinical trial data and the sufficiency of our cash, cash equivalents and investments to fund our operations, 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, 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, availability of funding sufficient for our foreseeable and unforeseeable operating expenses and capital expenditure requirements 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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