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

August 1, 2019

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

LEXINGTON, Mass.--(BUSINESS WIRE)--Aug. 1, 2019-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today reported financial results for the second quarter of 2019.

"As we move into the second half of the year, we are advancing our two wholly-owned clinical candidates for important chronic diseases that together affect millions of patients: CTP-543 for alopecia areata and CTP-692 for schizophrenia," said Roger D. Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "For CTP-543, we are focused on completing the necessary clinical and development activities for the briefing package that we plan to submit to FDA to support an end of Phase 2 meeting early next year. Given the favorable safety profile observed in Phase 1 testing for CTP-692, we are preparing for our Phase 2 efficacy study to begin in the fourth quarter of 2019."

Recent Business Highlights and Upcoming Milestones

CTP-543: a Janus kinase (JAK) inhibitor for the treatment of moderate-to-severe alopecia areata

- ***CTP-543 Third Dose, 12 mg Twice-Daily, Phase 2 Topline Data Expected Third Quarter of 2019.*** The Company expects to report topline results from the final cohort in its ongoing CTP-543 Phase 2 trial in the third quarter of 2019. The Phase 2 trial was designed to evaluate the safety and efficacy of three doses (4, 8, and 12 mg twice-daily) of CTP-543 compared to placebo in patients with moderate-to-severe alopecia areata. Interim results from the initial two cohorts for this dose-ranging trial, first reported in November 2018, showed that treatment with CTP-543 administered at an 8 mg twice-daily dose for 24 weeks met the primary endpoint with a statistically significant greater hair regrowth rate compared to placebo.
- ***CTP-543 Open Label Trial Evaluating 8 mg Twice-Daily vs. 16 mg Once-Daily Fully Enrolled.*** In May 2019, the Company announced that an open-label dose regimen trial evaluating CTP-543 for the treatment of alopecia areata at a dose of 8 mg twice-daily compared to a dose of 16 mg once-daily was fully enrolled. Topline results from the dose regimen trial are expected by year-end 2019. Data from this study, along with results from the Phase 2 dose-ranging trial, are intended to support an end of Phase 2 meeting with the U.S. Food and Drug Administration (FDA) in the first quarter of 2020 to review and discuss the Company's registration pathway for CTP-543 for the treatment of alopecia areata.
- ***CTP-543 Open Label Trial Evaluating 12 mg Twice-Daily vs. 24 mg Once-Daily Initiated.*** In May 2019,the Company initiated a second open label clinical trial to evaluate twice-daily compared to once-daily dosing of CTP-543 in patients with alopecia areata. Patients in this trial are randomized to receive either 12 mg twice-daily or 24 mg once-daily of CTP-543 over a 24 week treatment period. Topline data is expected in the first half of 2020.
- ***CTP-543 Open Label Extension Study Initiated April 2019.*** In April 2019, the Company initiated an open label extension study to continue to evaluate long-term safety and efficacy of CTP-543 in adult patients who complete 24 weeks of treatment in a previous qualifying CTP-543 clinical trial. Patients participating in the 12 mg cohort in the Phase 2 trial and the two open label dose regimen studies evaluating once-daily compared to twice-daily dosing, as well as future trials with CTP-543, are eligible to enroll in this study.
- ***The Company Filed Notice of Appeal in PTAB Proceeding.*** On June 7, 2019, the Company filed a Notice of Appeal with the Court of Appeals for the Federal Circuit in connection with the inter partes review (IPR) decision of the Company's U.S. Patent No. 9,249,149.

CTP-692: a deuterated form of D-serine for the adjunctive treatment of schizophrenia

- ***CTP-692 Phase 1 Positive Topline Results Reported.*** The safety assessments in the single- and multiple-ascending dose Phase 1 trials in healthy volunteers showed that CTP-692 was well tolerated over the dose ranges tested, which included the doses expected to be evaluated in Phase 2 testing. Importantly, key blood and urine markers of kidney function did not indicate any signs of renal impairment. These data are consistent with preclinical findings showing an improved renal safety profile for CTP-692 compared to non-deuterated D-serine which is known to produce renal toxicity in rats. Despite published clinical studies reporting the benefit of non-deuterated D-serine in the treatment of schizophrenia, its further development has been limited by renal safety concerns. The Company intends to advance CTP-692 into Phase 2 testing in the fourth quarter of 2019.
- ***CTP-692 Phase 1 Results to be Presented at ECNP Annual Congress.*** The Company will present its CTP-692 Phase 1 single- and multiple-ascending dose clinical results during a poster session at the European College of Neuropsychopharmacology Congress taking place September 7-10, 2019 in Copenhagen, Denmark.
- ***CTP-692 Poster Presentation at ASCP***. The initial Phase 1 CTP-692 trial evaluated the safety, tolerability, and

pharmacokinetics of a single oral dose of CTP-692 versus D-serine in a crossover study conducted in Australia. In the study, CTP-692 was shown to have increased plasma exposure compared to D-serine. In addition, CTP-692 was shown to be well tolerated in healthy volunteers. These results were presented at a poster session at The American Society of Clinical Psychopharmacology (ASCP) annual meeting, held May 28-31, 2019 in Scottsdale, AZ.

AVP-786 for Neurological Disorders

- ***Enrollment in the Second AVP-786 Phase 3 Alzheimer's Agitation Trial is Complete.*** In May 2019, Avanir Pharmaceuticals, a subsidiary of Otsuka Pharmaceuticals, announced that it completed enrollment in a second U.S. Phase 3 trial evaluating AVP-786 for the treatment of agitation in patients with dementia of the Alzheimer's type. The study is expected to complete in October 2019 and topline results are expected to be reported before year-end. Under a licensing agreement whereby Concert granted Avanir worldwide development and commercialization rights to AVP-786, Concert is eligible to receive additional milestone payments as well as tiered royalties on worldwide sales.

Second Quarter 2019 Financial Results

- ***Cash and Investment Position.*** Cash, cash equivalents and investments as of June 30, 2019, totaled $136.6 million. The Company expects its cash, cash equivalents and investments to be sufficient to fund the Company into the second half of 2020.
- ***Revenues.*** For the six months ended June 30, 2019, revenue was $1.1 million, compared to $10.5 million for the six months ended June 30, 2018. Revenue recognized in the 2019 and 2018 periods was due to upfront consideration for license arrangements with Cipla Technologies and Processa Pharmaceuticals, respectively.
- ***R&D Expenses.*** Research and development expenses were $14.5 million for the quarter ended June 30, 2019, compared to $8.9 million for the same period in 2018. For the six months ended June 30, 2019, research and development expenses were $30.3 million, compared to $17.5 million for the same period in 2018. The increase in research and development expenses relate primarily to the clinical development of CTP-543, including multiple ongoing Phase 2 clinical trials, 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 $5.0 million for the quarter ended June 30, 2019, compared to $5.5 million for the same period in 2018. For the six months ended June 30, 2019, G&A expenses were $10.6 million compared to $11.1 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 June 30, 2019, net loss applicable to common stockholders was $18.7 million, or $0.78 per share, compared with a net loss applicable to common stockholders of $13.3 million, or $0.57 per share, for the quarter ended June 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 second 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 the second quarter financial results 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 June 30,		Six Months Ended June 30,	
	2019	**2018**	**2019**	**2018**
Revenue:				

License and research and development revenue	$ 49	$ 2	$ 1,054	$ 10,481
Operating expenses:				
Research and development	14,496	8,862	30,286	17,518
General and administrative	4,978	5,514	10,587	11,144
Total operating expenses	19,474	14,376	40,873	28,662
Loss from operations	(19,425)	(14,374)	(39,819)	(18,181)
Investment income	883	660	1,750	1,300
Unrealized (loss) gain on marketable equity securities	(126)	669	(2,425)	(627)
Loss before tax provision	(18,668)	(13,045)	(40,494)	(17,508)
Provision for income taxes	—	280	—	280
Net Loss	$ (18,668)	$ (13,325)	$ (40,494)	$ (17,788)
Net loss per share applicable to common stockholders — basic and diluted	$ (0.78)	$ (0.57)	$ (1.71)	$ (0.76)
Weighted-average number of common shares used in net loss per share applicable to common stockholders— basic and diluted	23,790	23,402	23,650	23,313

Concert Pharmaceuticals, Inc.

Summary Balance Sheet Data

(in thousands)

	June 30, 2019	December 31, 2018
Cash and cash equivalents	$ 43,714	$ 17,770
Investments, available for sale	92,923	135,544
Working capital	137,912	171,400
Total assets	166,601	192,547
Deferred revenue	10,533	10,533
Total stockholders' equity	133,701	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 risks related to the clinical development of our therapeutic candidates and expectations regarding the sufficiency of our cash balance to fund operating expenses and capital expenditures, 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, 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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