Concert Pharmaceuticals Reports Second Quarter 2020 Financial Results and Provides Company Update

August 6, 2020

CTP-543 Phase 3 Trial Initiation in Alopecia Areata Expected to Begin Fourth Quarter 2020

CTP-692 Phase 2 Trial Enrollment in Schizophrenia Expected to be Complete by Year-End 2020

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

LEXINGTON, Mass.--(BUSINESS WIRE)--Aug. 6, 2020-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today reported financial results for the second quarter of 2020.

"We continue to be committed to advancing both of our lead programs as we embark on moving CTP-543 into pivotal trials. Our priority is to work with our clinical sites to support the safety and well-being of our trial participants, investigators and employees," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "We remain on track with our clinical program goals for CTP-543 in alopecia areata and CTP-692 in schizophrenia, both of which could potentially address important medical needs and sizeable market opportunities. For CTP-543, the Phase 3 program is expected to begin in the fourth quarter of 2020, supporting a potential new drug application to the FDA in 2023. For CTP-692, we continue to expect the Phase 2 trial will be fully enrolled by year-end with data expected in 2021."

Recent Business Highlights and Upcoming Milestones

CTP-543: An Investigational Treatment for Moderate-to-Severe Alopecia Areata

- ***Released CTP-543 Phase 2 SALT 20 Results.*** The Company released new data analyses from its completed Phase 2 dose-ranging study, including an analysis of patients achieving a SALT score ≤ 20 using the Severity of Alopecia Tool (SALT). Results showed that 26% and 42% of patients in the CTP-543 8 mg and 12 mg twice-daily dose groups, respectively, achieved a SALT score ≤ 20, showing statistically significant improvements of hair regrowth compared to placebo. A SALT score ≤ 20 corresponds to 80% or more hair coverage on the scalp. The Company plans to utilize a SALT score ≤ 20 as the primary endpoint in its Phase 3 program. The results were originally selected for an oral presentation at the Late-Breaking Research Program at the American Academy of Dermatology (AAD) Annual Meeting in March 2020; however, the in-person meeting was cancelled and the corresponding abstract for the CTP-543 results was published by AAD.
- ***CTP-543 Granted Breakthrough Therapy Designation for the Treatment of Alopecia Areata.*** In July 2020, the U.S. Food and Drug Administration (FDA) granted Breakthrough Therapy Designation for CTP-543, for the treatment of adult patients with moderate-to-severe alopecia areata. FDA Breakthrough Therapy Designation is intended to expedite the development and review of medicines aimed at treating a serious or life-threatening disease where there is preliminary clinical evidence that the investigational therapy may offer substantial improvement over existing therapies. There are currently no drugs approved by the FDA for the treatment of alopecia areata.
- ***PTAB Remand of IPR on Hold***. In January 2020, the U.S. Court of Appeals for the Federal Circuit (CAFC) granted Concert's motion to vacate and remand the previous inter partes review (IPR) decision by the Patent Trial and Appeal Board (PTAB) relating to Concert's Patent No. 9,249,149 in light of the recent CAFC ruling on the Constitution's Appointments Clause in *Arthrex, Inc. v. Smith & Nephew, Inc.*, 941 F.3d 1320 (Fed. Cir. 2019). In May 2020, the PTAB issued an Order putting on hold the reconsideration of any IPR proceedings, including Concert's IPR, that had been remanded by the CAFC under *Arthrex* until the Supreme Court acts on the petitions for certiorari that have been filed related to *Arthrex*.
- ***CTP-543 Phase 3 Program On Track to Begin in the Fourth Quarter 2020.*** The planned Phase 3 program to evaluate CTP-543 in patients with moderate-to-severe alopecia areata will include two randomized, double-blind, placebo-controlled clinical trials in the U.S., Canada and Europe. The first Phase 3 study is expected to begin in the fourth quarter of 2020.

CTP-692: An Investigational Adjunctive Treatment for Schizophrenia

- ***CTP-692 Phase 2 Enrollment On Track to Complete by Year-End 2020.*** The Company continues to expect to complete enrollment in the CTP-692 Phase 2 clinical trial by year-end 2020. The primary outcome measure is the change in the Positive and Negative Syndrome Scale (PANSS) total score at 12 weeks compared to baseline. Topline data is expected soon after trial completion.
- ***New CTP-692 Patent Provides Protection until 2038.*** In June 2020, the U.S. Patent and Trademark Office issued U.S. Patent No. 10,668,036 related to CTP-692. The patent, which will expire in 2038, covers methods of treating schizophrenia with CTP-692.
- ***CTP-692 Findings Presented at ASCP Virtual Meeting.*** CTP-692 Phase 1 clinical results and nonclinical results were

highlighted at the 2020 American Society of Clinical Psychopharmacology (ASCP) Virtual Annual Meeting on May 29-30. In a nonclinical study, CTP-692 was found to produce higher brain exposure compared to D-serine. 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 range tested, which include the doses being evaluated in the ongoing Phase 2 trial.

Second Quarter 2020 Financial Results

- ***Cash and Investment Position.*** Cash, cash equivalents and investments as of June 30, 2020 totaled $144.7 million as compared to $106.4 million as of December 31, 2019. Under its current operating plan, the Company expects its cash, cash equivalents and investments to fund the Company into the second half of 2021.
- ***Revenues.*** For the quarter ended June 30, 2020, revenue was $6.4 million. Revenue recognized during this period was the result of the expiration of licensing options under an existing collaboration with Celgene Corporation.
- ***R&D Expenses.*** Research and development expenses were $14.8 million for the quarter ended June 30, 2020, compared to $14.5 million for the same period in 2019. The increase in research and development expenses relates primarily to the clinical development program for CTP-692. R&D expenses are expected to increase in 2020 as the Company continues to develop CTP-692 in its Phase 2 clinical trial and prepares to advance CTP-543 into Phase 3 testing in the fourth quarter of 2020.
- ***G&A Expenses.*** General and administrative expenses were $4.7 million for the quarter ended June 30, 2020, compared to $5.0 million for the same period in 2019.
- ***Net Loss.*** For the quarter ended June 30, 2020, net loss applicable to common stockholders was $13.0 million, or $0.41 per share, as compared to net loss applicable to common stockholders of $18.7 million, or $0.78 per share, for the quarter ended June 30, 2019.

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)

(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2020	**2019**	**2020**	**2019**
Revenue:				
License and research and development revenue	$ 6,387	$ 49	$ 6,394	$ 1,054
Operating expenses:				
Research and development	14,788	14,496	28,774	30,286
General and administrative	4,731	4,978	9,403	10,587
Total operating expenses	19,519	19,474	38,177	40,873

Loss from operations	(13,132)	(19,425)	(31,783)	(39,819)
Investment income	355	883	918	1,750
Unrealized loss on marketable equity securities	(299)	(126)	(2,688)	(2,425)
Loss before income taxes	(13,076)	(18,668)	(33,553)	(40,494)
Income tax benefit	(85)	—	(85)	—
Net loss	$ (12,991)	$ (18,668)	$ (33,468)	$ (40,494)
Net loss per share applicable to common stockholders — basic and diluted	$ (0.41)	$ (0.78)	$ (1.11)	$ (1.71)
Weighted-average number of common shares used in net loss per share applicable to common stockholders— basic and diluted	31,455	23,790	30,283	23,650

Concert Pharmaceuticals, Inc.

Summary Balance Sheet Data

(in thousands)

(unaudited)

	June 30, 2020	December 31, 2019
Cash and cash equivalents	$ 27,094	$ 53,043
Investments, available for sale	117,623	53,395
Working capital	144,967	99,587
Total assets	171,999	137,471
Deferred revenue	4,163	10,533
Total stockholders' equity	144,085	101,457

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 patient enrollment and 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 and timing 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, expectations with respect to the protection of our intellectual property afforded by our patents 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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