Concert Pharmaceuticals Reports First Quarter 2019 Financial Results

May 2, 2019

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

LEXINGTON, Mass.--(BUSINESS WIRE)--May 2, 2019-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today reported financial results for the first quarter of 2019.

"The progress we are making to advance our pipeline positions us for a series of significant data readouts in 2019," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "Importantly, we expect data from Phase 2 trials of CTP-543 in alopecia areata later this year to support an end of Phase 2 meeting with FDA to discuss our registration strategy. In our schizophrenia program, the Phase 1 trials with CTP-692 will provide important safety data as we prepare to move the program into a Phase 2 trial in patients in the fourth quarter. Both of these clinical programs provide significant opportunities to address unmet medical needs for patients in sizeable markets."

Recent Business Highlights and Upcoming Milestones

CTP-543 for Alopecia Areata

The Company continues to make significant progress toward advancing CTP-543, a Janus kinase (JAK) inhibitor, for the treatment of moderate-to-severe alopecia areata, an autoimmune disease in which the immune system attacks hair follicles resulting in patchy or complete hair loss. Recent highlights include:

- ***Presented CTP-543 Phase 2 Interim Results at American Academy of Dermatology Annual Meeting.*** In March 2019, interim results from the Company's Phase 2 clinical trial evaluating CTP-543 in patients with moderate-to-severe alopecia areata were presented in an oral presentation during the late-breaking clinical trials session at the American Academy of Dermatology (AAD) Annual Meeting. The interim results 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 responder rate, compared to placebo. Regrowth of hair did not appear to plateau at Week 24. The primary endpoint measures the proportion of responders, defined as a ≥ 50% relative reduction in their overall Severity of Alopecia Tool (SALT) score from baseline. The responders in the 8 mg twice-daily dose group were evenly distributed among patients with patchy alopecia areata and the more severe forms with complete scalp baldness, alopecia totalis and alopecia universalis.
- ***CTP-543 Phase 2 Data Presented at World Congress for Hair Research.*** In April 2019, the Company presented its CTP-543 Phase 2 interim clinical data evaluating 4 mg and 8 mg twice daily in patients with alopecia areata in a poster presentation and in a sponsored lecture at the 11th World Congress for Hair Research (WCHR) in Barcelona.
- ***CTP-543 Phase 2 Trial for Alopecia Areata Fully Enrolled***. In January 2019, the Company completed patient enrollment of the final cohort evaluating 12 mg twice daily of CTP-543 in its Phase 2 trial for the treatment of moderate-to-severe alopecia areata. Data from the complete Phase 2 trial, including the 12 mg cohort, is expected in the third quarter of 2019.
- ***New CTP-543 Trial Initiated to Evaluate Once-Daily Dosing.*** In March 2019, Concertinitiated an open label clinical trial to evaluate once-daily compared to twice-daily oral dosing of CTP-543 in patients with alopecia areata. The trial, which is expected to complete in the fourth quarter of 2019, is intended to inform the optimal dosing regimen for CTP-543 for future clinical studies.
- ***PTAB Issues Final Written Decision in IPR Proceeding***. On April 8, 2019, the Patent Trial and Appeal Board (PTAB) of the U.S. Patent and Trademark Office issued a final written decision in connection with the inter partes review (IPR) of U.S. Patent No. 9,249,149 (the '149 patent). The PTAB found that the claims of the '149 patent are not patentable. The '149 patent claims cover the composition of matter of deuterated analogs of ruxolitinib, including CTP-543 which Concert is developing for alopecia areata. The Company intends to appeal the decision to the Federal Circuit. The '149 patent remains valid and enforceable until appeals have been exhausted. Importantly, this decision is specific to certain patent claims covering CTP-543 and does not affect other programs in our portfolio.

CTP-692 for Schizophrenia

CTP-692 is a deuterated form of D-serine, an endogenous human co-agonist of the N-methyl-D-aspartate (NMDA) receptor, that Concert is developing for the adjunctive treatment of schizophrenia. Recent highlights include:

- ***CTP-692 Crossover Results to be Presented 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 found to have increased plasma exposure compared to D-serine. In addition, CTP-692 was found to be well tolerated in healthy volunteers. These results will be presented during the poster session at The American Society of Clinical Psychopharmacology (ASCP) annual meeting, being held May 28-31, 2019 in Scottsdale, AZ.
- ***CTP-692 Phase 1 Single-Ascending Dose Trial Complete.*** In the first quarter of 2019, the Company completed a Phase 1 single-ascending dose trial evaluating the safety, tolerability, and pharmacokinetics of CTP-692 in healthy volunteers. The

Phase 1 single-ascending dose trial also evaluated the effect of food on the pharmacokinetics of the compound.

- ***CTP-692 Phase 1 Multiple-Ascending Dose Trial Underway.*** In April 2019, Concert initiated thePhase 1 multiple-ascending dose trial to evaluate the safety, tolerability, and pharmacokinetic profile of CTP-692 in healthy volunteers. This trial is a double-blind, placebo-controlled, multiple-ascending dose trial assessing CTP-692 dosed orally over seven consecutive days. Concert intends to report topline results from the single- and multiple-ascending dose Phase 1 trials in the second quarter of 2019.

AVP-786 for Neurological Disorders

AVP-786 is a combination of ultra-low dose quinidine and deuterium-modified dextromethorphan, which is being developed by Avanir Pharmaceuticals, a subsidiary of Otsuka Pharmaceuticals, under an exclusive license from Concert. Recent highlights include:

- ***AVP-786 First U.S. Phase 3 Trial Completed in February 2019.*** Avanir Pharmaceuticals, a subsidiary of Otsuka Pharmaceuticals, announced the completion of the first U.S. Phase 3 trial of AVP-786 for the treatment of agitation associated with Alzheimer's disease in February 2019. In March 2019, Otsuka announced that the trial, which used the Sequential Parallel Comparison Design (SPCD), demonstrated a statistically significant improvement on the primary endpoint on the Cohen-Mansfield Agitation Inventory for one of the two doses being evaluated; the other dose demonstrated numerical but not significant improvement on the SPCD analysis. Similar improvements were also observed on the key secondary endpoint. Avanir intends to publish the results in a peer-reviewed journal.
- ***AVP-786 Second U.S. Phase 3 Trial Expected to Complete in December 2019.*** A second U.S. Phase 3 trial evaluating AVP-786 for the treatment of agitation associated with Alzheimer's disease is ongoing and Avanir has stated that they expect to complete the trial in December 2019.
- ***AVP-786 Phase 2/3 Trial in Negative Symptoms of Schizophrenia Initiated.*** In April 2019, Avanir announced the initiation of a Phase 2/3 clinical trial to evaluate the effect of AVP-786 in treating negative symptoms of schizophrenia.

VX-561 for Cystic Fibrosis

In 2017, Vertex Pharmaceuticals acquired worldwide rights to VX-561 (formerly CTP-656) from Concert under an asset purchase agreement. If VX-561 is approved as part of a combination regimen to treat cystic fibrosis, Concert could receive up to an additional $90 million in milestones based on regulatory approval in the U.S. and reimbursement in the UK, Germany or France. Vertex recently announced that it has initiated two new trials in cystic fibrosis with VX-561:

- ***VX-561 Monotherapy Trial in Cystic Fibrosis Initiated.*** In the second quarter of 2019, Vertex initiated a Phase 2 dose-ranging study evaluating the once-daily potentiator VX-561 as a monotherapy as requested by the FDA. The study is designed to evaluate multiple doses of VX-561 to support potential Phase 3 development of VX-561 in a once-daily triple combination regimen.
- ***VX-561 Phase 2 Triple Combination Trial Initiated***. Vertex has initiated a Phase 2 study evaluating its next-generation corrector, VX-121, in combination with VX-561 and tezacaftor as a potential once-daily triple combination regimen.

Corporate Update

- ***Jesper Høiland Joins Board of Directors.*** In April 2019, Concert announced that Jesper Høiland has been appointed to its Board of Directors and will serve as a member of its Compensation Committee. Mr. Høiland is an industry veteran with more than 30 years of experience in the biopharmaceutical industry across numerous senior leadership roles, geographies and therapeutic areas. As President and Chief Executive Officer of Radius Health, Inc., Mr. Høiland successfully launched Radius' first commercial product, TYMLOS™. Prior to joining Radius, Mr. Høiland served as President and Executive Vice President of Novo Nordisk.

First Quarter 2019 Financial Results

- ***Cash and Investment Position.*** Cash, cash equivalents and investments as of March 31, 2019, totaled $153.8 million as compared to $153.3 million as of December 31, 2018. In the first quarter of 2019, Concert received $16.0 million initially held in escrow under the Asset Purchase Agreement with Vertex. Under its current operating plan, including the acceleration of CTP-543 into late stage development, the Company expects its cash, cash equivalents and investments to be sufficient to fund the Company into the second half of 2020.
- ***Revenues.*** Revenue for the quarter ended March 31, 2019 was $1.0 million, compared to $10.5 million for the same period in 2018. Revenue recognized in 2019 consists of a $1.0 million upfront payment from Cipla Technologies under a license agreement whereby Cipla has worldwide rights to develop and commercialize CTP-354, a novel $GABA_A$ receptor subtype-selective modulator. Under an existing CTP-354 agreement between Concert and the non-profit organization Fast Forward, the Company paid half of the upfront to Fast Forward. Revenue in the first quarter of 2018 relates primarily to the $10.5 million non-cash consideration received from Processa Pharmaceuticals under a licensing agreement whereby Processa has worldwide rights to develop and commercialize CTP-499.
- ***R&D Expenses.*** Research and development expenses were $15.8 million for the quarter ended March 31, 2019, compared to $8.7 million for the same period in 2018. The increase in R&D expenses relate primarily to the clinical

development of CTP-543, including multiple ongoing 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 substantially unchanged at $5.6 million for both the first quarter of 2019 and the same period of 2018. Decreases in employee-related expenses were offset by an increase in professional and legal expenses.
- ***Net Loss.*** For the quarter ended March 31, 2019, net loss applicable to common stockholders was $21.8 million, or $0.93 per share, compared with a net loss applicable to common stockholders of $4.5 million, or $0.19 per share, for the quarter ended March 31, 2018. The increase in net loss is a result of both higher R&D spending in the first quarter of 2019 compared to the first quarter of 2018, and higher revenues in 2018 due to an upfront payment from Processa.

Conference Call and Webcast

The Company will host a conference call and webcast today, Thursday, May 2, 2019, at 8:30 a.m. ET to provide an update on the Company and discuss first 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.

– Financial Tables to Follow –

Concert Pharmaceuticals, Inc.
Condensed Consolidated Statements of Operations
(in thousands, except per share amounts)

	Three Months Ended March 31,	
	2019	**2018**
Revenue:		
License and research and development revenue	$ 1,005	$ 10,479
Operating expenses:		
Research and development	15,790	8,656
General and administrative	5,609	5,630
Total operating expenses	21,399	14,286
Loss from operations	(20,394)	(3,807)
Investment income	867	640
Unrealized loss on marketable equity securities	(2,299)	(1,296)
Net loss	(21,826)	(4,463)
Net loss per share applicable to common stockholders - basic and diluted	$ (0.93)	$ (0.19)
Weighted average shares outstanding - basic and diluted	23,508	23,223

Concert Pharmaceuticals, Inc.
Summary Balance Sheet Data
(in thousands)

	March 31, 2019	**December 31, 2018**
Cash and cash equivalents	$ 60,262	$ 17,770
Investments, available for sale	93,545	135,544
Working capital	153,869	171,400
Total assets	183,391	192,547
Deferred revenue	10,533	10,533
Total stockholders' equity	149,798	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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