Concert Pharmaceuticals Reports Year Ended 2018 Financial Results and Provides Update on Clinical Programs

February 28, 2019

Significant Clinical Progress in 2018; Multiple Trials with Milestones in 2019

Additional CTP-543 Phase 2 Data Expected Q3 2019 for Treatment of Alopecia Areata

CTP-692 Poised to Advance into Phase 2 Development by Year End 2019 as Adjunctive Treatment for Schizophrenia

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

LEXINGTON, Mass.--(BUSINESS WIRE)--Feb. 28, 2019-- Concert Pharmaceuticals, Inc. (NASDAQ:CNCE) today reported financial results for the year ended December 31, 2018, as well as provided an update on its product pipeline and corporate activities.

"In 2019, we are focused on advancing our proprietary clinical candidates CTP-543 for alopecia areata and CTP-692 for schizophrenia, with key readouts on the horizon for both programs. For CTP-543, based on positive data from the interim analysis of our Phase 2 trial, we believe we may have the opportunity to move more quickly into pivotal trials than previously projected, with the goal of developing CTP-543 as a first-in-class oral treatment for alopecia areata," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "For CTP-692, we are enthusiastic about the rapid progress of this program and its potential as a truly novel approach in the treatment of schizophrenia. Our Phase 2 trial, which is intended to support advancement into registration trials, is expected to begin later this year. CTP-692 is another example of the potential of our technology to create innovative medicines with differentiated properties that can have meaningful impact for patients."

Dr. Tung added, "In addition to our proprietary clinical development programs, our collaborator Avanir Pharmaceuticals, a subsidiary of Otsuka Pharmaceuticals, expects to complete the first Phase 3 trial evaluating AVP-786 for Alzheimer's agitation in April 2019, with data readout soon thereafter."

Recent Business Highlights and Upcoming Milestones

CTP-543 for Alopecia Areata

The Company continues to make significant progress toward advancing CTP-543 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:

- ***Positive Interim Phase 2 CTP-543 Data Supports Advancement into Late Stage Trials.*** In November 2018, Concert announced interim topline results from the first two cohorts of the Phase 2 trial showing that patients treated with an 8 mg twice-daily dose of CTP-543 for 24 weeks met the primary efficacy endpoint compared to placebo ($p < 0.001$). Regrowth of hair did not appear to plateau at Week 24. The primary outcome measure is the proportion of patients with a ≥ 50% relative reduction in the overall Severity of Alopecia Tool (SALT) score between Week 24 and baseline. Treatment with CTP-543 was generally well tolerated and no serious adverse events were reported. These data will be presented at the Late-Breaking Research Program during the American Academy of Dermatology (AAD) Annual Meeting on March 2, 2019.
- ***Enrollment Completed in Final Cohort of CTP-543 Phase 2 Trial.*** In January 2019, the Company completed enrollment of the final cohort of its Phase 2 clinical trial evaluating CTP-543. All patients who complete treatment in the 12 mg cohort (12 mg twice daily vs placebo twice daily) will be eligible to enroll into an open-label extension study whereby all patients will receive CTP-543. The Company expects to report topline data from the Phase 2 trial, including the 12 mg cohort, in the third quarter of 2019.
- ***CTP-543 Dose Regimen Trial Planned.*** In the first quarter of 2019, the Company intends to initiate an open label trial to evaluate once-daily dosing compared to twice-daily dosing of CTP-543 in patients with alopecia areata. The trial is intended to inform an optimal dose regimen to be evaluated in future clinical trials. All patients who complete 24 weeks of treatment in the dose regimen trial will be eligible to enroll into an open-label extension study. The dose regimen trial is expected to be completed in the second half of 2019.
- ***CTP-543 Food Effect Trial Planned***. In the first half of 2019, the Company intends to conduct a food effect trial to assess the relative bioavailability of oral doses of CTP-543 under fasted and fed conditions in 14 healthy volunteers.
- ***PTAB Decision on IPR Expected April 2019.*** In January 2019, the Patent Trial and Appeal Board (PTAB) heard oral arguments in an inter partes review (IPR) proceeding challenging the validity of Concert's U.S. Patent No. 9,249,149. The PTAB is expected to render a final written decision in the IPR by April 9, 2019. An unfavorable outcome in the IPR proceeding would not prohibit Concert from developing CTP-543 for alopecia areata, and the Company's development timelines for CTP-543 remain on track.

CTP-692 for Schizophrenia

In 2018, the Company advanced CTP-692 from preclinical testing into first-in-human evaluation with plans to commence Phase 2 testing in the fourth quarter of 2019. CTP-692 is a deuterated form of D-serine, an endogenous 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 Phase 1 Crossover Study Completed***. The Phase 1 crossover study evaluated the safety, tolerability, and pharmacokinetics of a single oral dose of CTP-692 versus D-serine. In individuals treated with both compounds, CTP-692 was found to have increased plasma exposure compared to D-serine. In addition, CTP-692 was found to be generally well tolerated in healthy volunteers and no serious adverse events were reported.
- ***CTP-692 Phase 1 Single and Multiple Ascending Dose Trials.*** In January 2019, Concert initiated a Phase 1 single ascending dose trial under a U.S. Investigational New Drug (IND) application to evaluate the safety, tolerability, and pharmacokinetic profile of CTP-692 in healthy volunteers. In addition, the Phase 1 program will include a study to assess multiple doses of CTP-692 dosed orally over seven days in a double-blind, placebo-controlled, multiple-ascending dose trial. Topline data from the Phase 1 program, including readout of renal safety markers, is expected in the first half of 2019.
- ***CTP-692 Phase 2 Trial Planned in the Fourth Quarter of 2019.*** Following the successful completion of the ongoing CTP-692 Phase 1 program, the Company intends to advance CTP-692 into a Phase 2 trial in the fourth quarter of 2019 that is intended to support advancement into pivotal evaluation for the adjunctive treatment of schizophrenia.
- ***CTP-692 Preclinical Results Presented at American College of Toxicology Annual Meeting.*** In November 2018, Concert presented preclinical results that support the potential of CTP-692 to improve upon the safety profile of D-serine. In preclinical evaluation, Concert demonstrated that selective deuterium modification resulted in increased exposure of CTP-692 compared to a similar dose of D-serine. Unlike D-serine, CTP-692 did not cause undesirable changes in important markers of kidney function.

Collaborations

- ***AVP-786 U.S. Phase 3 Trials Expected to Complete in 2019.*** Avanir Pharmaceuticals, a subsidiary of Otsuka Pharmaceuticals, plans to complete the first Phase 3 trial of AVP-786 for the treatment of agitation associated with Alzheimer's disease in April 2019 and release the topline results promptly thereafter. In January 2019, Avanir announced that enrollment in the trial had been completed. 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.
- ***Cipla to Develop $GABA_A$ Modulator CTP-354.*** In the first quarter of 2019, Concert granted Cipla Technologies LLC an exclusive worldwide license to develop and commercialize CTP-354, a novel $GABA_A$ receptor subtype-selective modulator. Building on Concert's initial preclinical and clinical evaluation, Cipla intends to develop CTP-354 for the treatment of spasticity. Concert received an upfront payment and has the potential to receive future milestones as well as royalties on future product sales.

Full Year 2018 Financial Results

- ***Cash and Investment Position***. Cash, cash equivalents and investments as of December 31, 2018, totaled $153.3 million as compared to $203.2 million as of December 31, 2017. 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 was $10.5 million for the year ended December 31, 2018, compared to $143.9 million for the year ended December 31, 2017. Revenue recognized in 2018 consists of $10.5 million in non-cash consideration received from Processa Pharmaceuticals under a licensing agreement whereby Processa has worldwide rights to develop and commercialize CTP-499. Revenue in 2017 relates to the asset purchase agreement whereby Vertex Pharmaceuticals acquired CTP-656. As part of the agreement, Vertex paid Concert $160 million in cash for worldwide development and commercialization rights to CTP-656.
- ***R&D Expenses.*** Research and development expenses were $43.1 million for the year ended December 31, 2018, compared to $30.2 million for the year ended December 31, 2017, an increase of $12.9 million. The increase in external expenses in 2018 was to support clinical trial and manufacturing costs in CTP-543 and CTP-692, respectively.
- ***G&A Expenses.*** General and administrative expenses were $22.9 million for the year ended December 31, 2018, compared to $21.0 million for the year ended December 31, 2017, an increase of $1.9 million. The increase in general and administrative expenses was primarily attributable to non-cash stock-based compensation.
- ***Net (Loss) Income.*** For the year ended December 31, 2018, net loss applicable to stockholders was $56.0 million, or $2.40 per share, as compared to net income applicable to stockholders of $95.6 million, or $4.20 per share for the year ended December 31, 2017.

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 full year 2018 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 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 December 31,		Twelve Months Ended December 31,	
	2018	**2017**	**2018**	**2017**
Revenue:				
License and research and development revenue	$ 13	$ 10	$ 10,505	$ 62
Other revenue	—	2	—	143,829
Total revenue	13	12	10,505	143,891
Operating expenses:				
Research and development	14,560	7,565	43,149	30,223
General and administrative	5,516	5,184	22,940	21,019
Total operating expenses	20,076	12,749	66,089	51,242
(Loss) Income from operations	(20,063)	(12,737)	(55,584)	92,649
Interest and other (expense) income, net	(771)	4,194	(127)	2,690
(Loss) Income before tax provision	(20,834)	(8,543)	(55,711)	95,339
Provision (Benefit) for income taxes	15	(2,477)	313	(300)
Net (loss) income	$ (20,849)	$ (6,066)	$ (56,024)	$ 95,639
Net (loss) income attributable to common stockholders - basic	(20,849)	(6,066)	(56,024)	95,195
Net (loss) income attributable to common stockholders - diluted	(20,849)	(6,066)	(56,024)	95,210
Net (loss) income per share applicable to common stockholders				
Basic	$ (0.89)	$ (0.26)	$ (2.40)	$ 4.20
Diluted	$ (0.89)	$ (0.26)	$ (2.40)	$ 4.06
Weighted-average number of common shares used in net (loss) income per share applicable to common stockholders:				
Basic	23,432	22,909	23,370	22,641
Diluted	23,432	22,909	23,370	23,442

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

	December 31, 2018	**December 31, 2017**
Cash and cash equivalents	$ 17,770	$ 27,665
Investments, available for sale	135,544	175,500
Working capital	171,400	199,289
Total assets	192,547	211,736
Deferred revenue	10,533	10,301
Total stockholders' equity	167,740	196,432

Additional information about Concert's pipeline product, CTP-543 and CTP-692 and their therapeutic indications, are available online at: https://www.concertpharma.com/product-pipeline/

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.comor 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 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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Source: Concert Pharmaceuticals, Inc.

Justine Koenigsberg (Investors)
Concert Pharmaceuticals, Inc.
(781) 674-5284
ir@concertpharma.com

Kathryn Morris (Media)
The Yates Network
(914) 204-6412
kathryn@theyatesnetwork.com