

Concert Pharmaceuticals Reports First Quarter 2018 Financial Results

May 3, 2018

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

LEXINGTON, Mass.--(BUSINESS WIRE)--May 3, 2018-- [Concert Pharmaceuticals, Inc.](#) (NASDAQ: CNCE) today reported financial results for the first quarter of 2018.

"Concert has begun 2018 with strong momentum as our pipeline of first-in-class drug candidates for serious diseases is progressing in important efficacy trials, with the first data read out later this year for CTP-543," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "We look forward to the progression of multiple Concert-discovered compounds over the coming quarters, including our proprietary drug candidates, CTP-543 for alopecia areata and CTP-692 for schizophrenia."

Dr. Tung also commented, "As we've demonstrated with our platform, early clinical studies of well-chosen deuterium-modified drugs can rapidly create value. Two clear examples of that include VX-561, which Vertex may advance into Phase 3 development for cystic fibrosis, and AVP-786 which is in Phase 3 evaluation for Alzheimer's agitation under our collaboration with Avanir Pharmaceuticals. Both VX-561 and AVP-786 have the potential to provide substantial additional financial upside to Concert."

Recent Business Highlights and Upcoming Milestones

Autoimmune Dermatology

- **CTP-543 Phase 2a Trial for Alopecia Areata Fully Enrolled.** In April 2018, the Company completed patient enrollment of its Phase 2a trial evaluating CTP-543 for the treatment of moderate-to-severe alopecia areata. This is a double-blind, randomized, placebo-controlled, sequential dose trial to evaluate the safety and efficacy of two doses twice daily of CTP-543 in approximately 90 patients. Topline data from the 4 mg and 8 mg cohorts is expected in the fourth quarter of 2018. The primary outcome measure will utilize the severity of alopecia tool (SALT) after 24 weeks of dosing. If appropriate, the protocol may be amended to explore 12 mg twice daily of CTP-543.
- **CTP-543 Granted Fast Track Designation.** In January 2018, the Company announced that the U.S. Food and Drug Administration (FDA) granted Fast Track designation for CTP-543. Fast Track designation is intended to facilitate the development and expedite the review process for therapies for serious medical conditions which offer the potential to significantly advance the existing standard of care.
- **FDA Voice of the Patient: Alopecia Areata.** Following the U.S. Food and Drug Administration's (FDA) Patient-Focused Drug Development meeting held in September 2017 on alopecia areata, the FDA summarized the input shared by patients and patient representatives in a [Voice of the Patient](#) report. The public meeting on alopecia areata provided FDA with patient input on the impact of alopecia areata, including on daily life, patient views on treatment approaches, and decision factors taken into account when selecting a treatment.
- **PTAB Institutes IPR Proceeding.** In April 2018, the Patent Trial and Appeal Board (PTAB) granted the request for rehearing of the PTAB's decision denying institution of the Inter Partes Review (IPR) filed by Incyte Corporation against Concert's U.S. Patent No. 9,249,149. Concert believes in the validity of its patent claims and intends to vigorously defend the patent.

Neuropsychiatry

- **CTP-692 Preclinical Results Support Development in Schizophrenia.** In March 2018, Concert announced the selection of CTP-692, a novel drug candidate for adjunctive treatment of schizophrenia, as its next development candidate. CTP-692 is a deuterated form of D-serine, an endogenous co-agonist of the NMDA receptor. Patients with schizophrenia have been shown to have lower plasma and cerebrospinal fluid concentrations of D-serine than individuals without schizophrenia. Based on early clinical results of D-serine and Concert's initial preclinical assessment of CTP-692, the Company believes that CTP-692 has the potential to improve clinical outcomes in patients with schizophrenia. The Company intends to advance CTP-692 into clinical development by year-end 2018.
- **CTP-692 NMDA Receptor Activity Nearly Identical to D-Serine.** In preclinical testing, Concert demonstrated that CTP-692 and D-serine have nearly identical binding and functional activity at the human NMDA receptor.

First Quarter 2018 Financial Results

- **Cash and Investments Position:** Cash, cash equivalents and investments as of March 31, 2018, totaled \$191.0 million as compared to \$203.2 million as of December 31, 2017. Concert expects its cash, cash equivalents and investments as of March 31, 2018 to be sufficient to fund the Company into 2021.
- **Revenues:** Revenue was \$10.5 million for the quarter ended March 31, 2018, compared to \$20,000 for the same period in 2017. Revenue recognized in 2018 consists of \$10.5 million primarily in non-cash consideration received from Processa Pharmaceuticals under a licensing agreement whereby Processa has worldwide rights to develop and commercialize CTP-499, a deuterated analog of 1-(S)-5-hydroxyhexyl-3,7-dimethylxanthine, an active metabolite of pentoxifylline.
- **R&D Expenses:** Research and development expenses were \$8.7 million for the quarter ended March 31, 2018, compared to \$8.2 million for the same period in 2017. In the first quarter of 2018, research and development expenses were primarily associated with the development of CTP-543 and CTP-692. In the first quarter of 2017, research and development expenses

were primarily associated with the development of CTP-543 and CTP-656, the Company's product candidate for cystic fibrosis which was acquired by Vertex Pharmaceuticals in July 2017.

- **G&A Expenses:** General and administrative expenses were \$5.6 million for the quarter ended March 31, 2018, compared to \$5.3 million for the same period in 2017. The increase in general and administrative expenses was primarily related to an increase in non-cash stock-based compensation expense.
- **Net Loss:** For the quarter ended March 31, 2018, net loss applicable to common stockholders was \$4.5 million, or \$0.19 per share, compared with a net loss applicable to common stockholders of \$13.3 million, or \$0.60 per share, for the quarter ended March 31, 2017.

Conference Call and Webcast

The Company will host a conference call and [webcast](#) today, Thursday, May 3, 2018, 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)

	Quarter Ended March 31, 2018	2017
Revenue:		
License and research and development revenue	\$ 10,479	\$ 20
Operating expenses:		
Research and development	8,656	8,237
General and administrative	5,630	5,253
Total operating expenses	14,286	13,490
Loss from operations	(3,807)	(13,470)
Investment income	640	137
Unrealized loss on marketable equity securities	(1,296)	—
Net loss	\$ (4,463)	\$ (13,333)
Net loss per share applicable to common stockholders — basic and diluted	\$ (0.19)	\$ (0.60)
Weighted-average number of common shares used in net loss per share applicable to common stockholders— basic and diluted	23,223	22,377

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

	March 31, 2018	December 31, 2017
Cash and cash equivalents	\$ 35,697	\$ 27,665
Investments, available for sale	155,305	175,500
Working capital	212,893	199,289
Total assets	227,646	211,736
Deferred revenue	10,533	10,301
Total stockholders' equity	\$ 210,397	\$ 196,432

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 address unmet patient needs. The Company's approach starts with starts with previously studied compounds, including approved drugs, in which deuterium substitution has the potential to enhance clinical safety, tolerability or efficacy. Concert has a [broad pipeline](#) of innovative medicines targeting autoimmune and inflammatory 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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