

Concert Pharmaceuticals Reports Year Ended 2017 Financial Results

March 1, 2018

CTP-543 On Track to Report Topline Phase 2a Results in Alopecia Areata by Year-End

New Neuropsychiatric Product Candidate Poised to Enter Clinical Development in 2018

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

LEXINGTON, Mass.--(BUSINESS WIRE)--Mar. 1, 2018-- [Concert Pharmaceuticals, Inc.](#) (NASDAQ:CNCE) today reported financial results for the year ended December 31, 2017, as well as provided an update on its product pipeline and corporate activities.

"Our focus for Concert in 2018 centers around pipeline advancement, notably CTP-543 for moderate-to-severe alopecia areata, and our newly announced development candidate, CTP-692, for adjunctive treatment of schizophrenia. These differentiated product candidates were created with our deuterium platform to address important unmet patient needs," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "We believe that we have numerous ways to further grow our pipeline and we will be exploring additional opportunities to do so throughout 2018."

Dr. Tung added, "The achievements we realized in 2017, including the CTP-656, now VX-561, asset purchase agreement with Vertex and the Patent Trial and Appeal Board decision upholding the validity of our CTP-543 patent, position us well to capitalize on our product candidate opportunities and continue to validate our deuterium approach."

Recent Business Highlights and Upcoming Milestones

- **Enrollment in Second Cohort of CTP-543 Phase 2a Trial Initiated.** In February 2018, the Company initiated enrollment of the second cohort of its Phase 2a clinical trial evaluating CTP-543. Concert is developing 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. The Company expects to report topline data from the 4 mg and 8 mg cohorts of the Phase 2a trial in the fourth quarter of 2018.
- **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 reserved for therapies for serious medical conditions which offer the potential to significantly advance the existing standard of care, and is designed to facilitate their development and expedite the review process.
- **CTP-543 Patent Proceedings.** In October 2017, the Patent Trial and Appeal Board (PTAB) denied Incyte's petition to institute inter partes review (IPR) of Concert's U.S. Patent No. 9,249,149. The denial of Incyte's IPR petition upholds the validity of Concert's composition of matter patent claims covering CTP-543. In January 2018, the PTAB denied Concert's petition to institute Post Grant Review (PGR) of U.S. Patent No. 9,662,335 assigned to Incyte. The January PTAB decision does not prohibit Concert from developing CTP-543 for alopecia areata and the development timelines for CTP-543 remain on track.
- **CTP-692 Selected as New Development Candidate.** Concert selected 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 N-methyl-D-aspartate (NMDA) receptor. Based on the clinically demonstrated effects of d-serine and our initial preclinical assessment of CTP-692, the Company believes that CTP-692 has the potential to help restore NMDA receptor activity in key areas of the brain to improve clinical outcomes in patients with schizophrenia. The Company intends to conduct preclinical evaluation and advance CTP-692 into clinical development by year-end 2018.
- **Marc Becker Named Chief Financial Officer.** Effective January 4, 2018, Marc Becker joined Concert as Chief Financial Officer. Mr. Becker is a seasoned financial executive with more than 15 years of experience in the biotechnology industry. Prior to joining Concert, he was Senior Vice President and Chief Financial Officer at CRISPR Therapeutics.
- **NASDAQ Biotechnology Index.** In December 2017, Concert was selected for addition to the NASDAQ Biotechnology Index. The NASDAQ Biotechnology Index is designed to track the performance of a set of NASDAQ-listed securities that are classified as either biotechnology or pharmaceutical according to the Industry Classification Benchmark.

Full Year 2017 Financial Results

- **Cash and Investments Position.** Cash, cash equivalents and investments as of December 31, 2017, totaled \$203.2 million as compared to \$96.2 million as of December 31, 2016. Concert expects its cash, cash equivalents and investments as of December 31, 2017 to be sufficient to fund the Company into 2021.
- **Revenues.** Revenue was \$143.9 million for the year ended December 31, 2017, compared to \$174,000 for the year ended December 31, 2016. The increase in revenue relates to the asset purchase agreement with Vertex Pharmaceuticals whereby Vertex acquired worldwide development and commercialization rights to CTP-656 and other assets related to the treatment of cystic fibrosis for \$160 million.
- **R&D Expenses.** Research and development expenses were \$30.2 million for the year ended December 31, 2017, compared to \$37.0 million for the year ended December 31, 2016, a decrease of \$6.8 million. The decrease was primarily due to the discontinuation of CTP-656 as a result of the asset purchase agreement with Vertex.
- **G&A Expenses.** General and administrative expenses were \$21.0 million for the year ended December 31, 2017, compared to \$14.4 million for the year ended December 31, 2016, an increase of \$6.6 million. The increase in general and administrative expenses was primarily related to an increase in stock compensation as well as professional and legal fees in connection with

the CTP-656 asset purchase agreement and intellectual property matters related to CTP-543.

- **Net Income (Loss).** For the year ended December 31, 2017, net income was \$95.6 million, or \$4.20 per share, as compared to a net loss of \$50.7 million, or \$2.28 per share for the year ended December 31, 2016.

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 2017 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.

- Financial Tables to Follow -

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2017	2016	2017	2016
Revenue:				
License and research and development revenue	\$ 10	\$ 21	\$ 62	\$ 174
Other Revenue	2	—	143,829	—
Total revenue	12	21	143,891	174
Operating expenses:				
Research and development	7,565	8,658	30,223	36,983
General and administrative	5,184	3,515	21,019	14,358
Total operating expenses	12,749	12,173	51,242	51,341
Income (loss) from operations	(12,737)	(12,152)	92,649	(51,167)
Interest and other income, net	4,194	109	2,690	447
Income (loss) before tax (provision) benefit	(8,543)	(12,043)	95,339	(50,720)
Benefit from income taxes	(2,477)	—	(300)	—
Net income (loss)	\$ (6,066)	\$ (12,043)	\$ 95,639	\$ (50,720)
Net income (loss) attributable to common stockholders - basic	(6,066)	(12,043)	95,195	(50,720)
Net income (loss) attributable to common stockholders - diluted	(6,066)	(12,043)	95,210	(50,720)
Net income (loss) per share attributable to common stockholders:				
Basic	\$ (0.26)	\$ (0.54)	\$ 4.20	\$ (2.28)
Diluted	\$ (0.26)	\$ (0.54)	\$ 4.06	\$ (2.28)
Weighted-average number of common shares used in net income (loss) per share attributable to common stockholders:				
Basic	22,909	22,287	22,641	22,233
Diluted	22,909	22,287	23,442	22,233

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

	December 31, 2017	December 31, 2016
Cash and cash equivalents	\$ 27,665	\$ 40,555
Investments, available for sale	175,500	55,630
Working capital	199,289	92,159
Total assets	211,736	100,395
Deferred revenue	10,301	10,050
Total stockholders' equity	\$ 196,432	\$ 85,594

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 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 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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