Concert Pharmaceuticals Reports Second Quarter 2022 Financial Results

August 4, 2022

CTP-543 Positive Topline Results Reported in THRIVE-AA1 and THRIVE-AA2 Phase 3 Studies in Alopecia Areata

CTP-543 Has the Potential to Address an Important Unmet Need for a Large, Underserved Patient Population

Conference Call Scheduled Today at 8:30 a.m. ET; Online Registration Required to Access Dial in and PIN for Live Call to Ask Questions

LEXINGTON, Mass.--(BUSINESS WIRE)--Aug. 4, 2022-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today reported financial results for the second quarter of 2022.

"We are very happy to have seen consistent efficacy and safety results across our two Phase 3 clinical trials of CTP-543 in alopecia areata. The positive results that we reported from THRIVE-AA1 in May and from THRIVE-AA2 earlier this week, will form the basis of our New Drug Application, which we expect to submit to the FDA in the first half of 2023," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "We have continued to execute well on a development program for CTP-543 that provides a robust data package in support of a potential FDA approval. We are proud that CTP-543 could offer a near-term and potentially best-in-class treatment option for adults with moderate to severe alopecia areata."

Recent Highlights and Upcoming Milestones
CTP-543: An Investigational Treatment for Moderate to Severe Alopecia Areata

- ***Positive Phase 3 Results Reported for CTP-543 THRIVE-AA1 Study.*** In May 2022, the Company reported positive topline results for its first CTP-543 Phase 3 clinical trial. A statistically significant proportion of patients treated with either 8 mg twice-daily or 12 mg twice-daily of CTP-543 in the THRIVE-AA1 study experienced greater scalp regrowth compared to placebo. The proportion of patients achieving a Severity of Alopecia Tool (SALT) score of 20 or less (meaning 20 percent or less scalp hair loss) at Week 24 was 41.5 percent in the 12 mg twice-daily dose group and 29.6 percent in the 8 mg twice-daily dose group, compared to 0.8 percent of patients in the placebo group. The treatment difference for both dose groups of CTP-543 relative to placebo was statistically significant ($p<0.0001$). The safety profile seen with CTP-543 in THRIVE-AA1 was consistent with previous studies of CTP-543.
- ***Positive Phase 3 Results Reported for CTP-543 THRIVE-AA2 Study.*** In August 2022, the Company reported positive topline results for its second CTP-543 Phase 3 clinical trial. A statistically significant proportion of patients treated with either 8 mg twice-daily or 12 mg twice-daily of CTP-543 in the THRIVE-AA2 study experienced greater scalp regrowth compared to placebo. The proportion of patients achieving a SALT score of 20 or less at Week 24 was 38.3 percent in the 12 mg twice-daily dose group and 33.0 percent in the 8 mg twice-daily dose group, compared to 0.8 percent of patients in the placebo group. The treatment difference for both dose groups of CTP-543 relative to placebo was statistically significant ($p<0.0001$). The safety profile seen with CTP-543 in THRIVE-AA2 was consistent with previous studies of CTP-543.
- ***JAAD Publication Highlights Significant Reduction in Severity of Hair Loss with CTP-543.*** The Company recently published safety and efficacy data from its randomized, double-blind, placebo-controlled dose-ranging Phase 2 clinical trial of CTP-543 in the *Journal of the American Academy of Dermatology (JAAD)*. The publication reported clinically meaningful and statistically significant scalp hair regrowth after 24 weeks of treatment with CTP-543 in both the 8 mg twice-daily and 12 mg twice-daily dose groups in patients with alopecia areata, as well as safety data and patient-reported outcomes of improvement. This trial supported the advancement of CTP-543 into Phase 3 development.
- ***Concert to Participate in Alopecia Areata Awareness Month in September.*** Throughout the month of September, Concert, along with the alopecia areata community, will raise awareness and recognize the importance of alopecia areata, a serious autoimmune disorder that affects up to approximately 1.5 million individuals in the U.S. and which often results in poor health-related quality of life as well as high incidence of anxiety and depression. Follow our #LightItUpBlue4AlopeciaAreata campaign on Twitter at @ConcertPharma.

Second Quarter 2022 Financial Results

- ***Cash and Investment Position.*** Cash, cash equivalents and investments as of June 30, 2022 totaled $153.7 million as compared to $141.6 million as of December 31, 2021. Under its current operating plan, the Company expects its cash, cash equivalents and investments to fund the Company into the second quarter of 2023. In June 2022, Concert closed an equity offering raising gross proceeds of $54.6 million before underwriting discounts and offering expenses. Concurrent with the initial closing of the offering, Concert received $18.9 million from the partial exercise of the Tranche 1 warrants issued to BVF Partners L.P. and RA Capital Management in connection with its November 2021 financing. The Company has the potential to receive an additional $70.1 million in 2022 upon full exercise of the remaining warrants issued in connection with the November 2021 financing.

- ***R&D Expenses*.** Research and development expenses were $20.9 million for the quarter ended June 30, 2022, compared to $20.2 million for the same period in 2021. The increase in research and development expenses relates primarily to the clinical development program for CTP-543.
- ***G&A Expenses*.** General and administrative expenses were $4.8 million for the quarter ended June 30, 2022, compared to $5.6 million for the same period in 2021. The decrease in general and administrative expenses relates primarily to decreased non-cash stock-based compensation.
- ***Net (Loss) Income.*** For the quarter ended June 30, 2022, net loss applicable to common stockholders was $24.0 million, or $0.59 per share, as compared to net income applicable to common stockholders of $5.4 million, or $0.16 per share, for the quarter ended June 30, 2021. Net income for the quarter ended June 30, 2021 included $32.0 million in revenue from proceeds received from Vertex Pharmaceuticals, Inc. for the purchase of potential future milestones under the companies' 2017 asset purchase agreement related to VX-561.

Conference Call and Webcast: *New System to Access Call Live and On Demand*
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.

Please note that there is a new system to access the live call in order to ask questions. To join the live call, please register here. A dial in and unique PIN number will be provided to join the call.

An audio-only webcast of the call may be accessed in the Investors section of the Company's website at www.concertpharma.com. 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)

(unaudited)

	Three Months Ended June 30,		Six Months Ended June 30,	
	2022	**2021**	**2022**	**2021**
Revenue:				
License and research and development revenue	$ 21	$ 17	$ 22	$ 22
Other revenue	—	32,000	—	32,000
Total revenue	21	32,017	22	32,022
Operating expenses:				
Research and development	20,855	20,184	51,344	38,684
General and administrative	4,849	5,614	10,389	11,099
Total operating expenses	25,704	25,798	61,733	49,783
(Loss) income from operations	(25,683)	6,219	(61,711)	(17,761)

Investment income	145	15	165	40
Unrealized (loss) gain on marketable equity securities	(60)	(809)	(624)	477
Unrealized gain on warrant liabilities	2,050	—	894	—
Net (loss) income	$ (23,548)	$ 5,425	$ (61,276)	$ (17,244)
Net (loss) income attributable to common stockholders — basic and diluted	(24,045)	5,415	(61,773)	(17,244)
Net (loss) income per share applicable to common stockholders — basic and diluted	$ (0.59)	$ 0.16	$ (1.59)	$ (0.51)
Weighted-average number of common shares used in net (loss) income per share applicable to common stockholders:				
Basic	41,042	33,974	38,877	33,934
Diluted	41,042	34,083	38,877	33,934

Concert Pharmaceuticals, Inc.

Summary Balance Sheet Data

(in thousands)

(unaudited)

	June 30, 2022	**December 31, 2021**
Cash and cash equivalents	$ 139,245	$ 141,636
Investments, available for sale	14,488	—
Working capital	145,956	134,209
Total assets	176,393	165,316
Deferred revenue	7,595	7,595
Total stockholders’ equity	127,792	112,225

About Concert

Concert Pharmaceuticals is a late-stage clinical biopharmaceutical company that is developing small molecule drugs that it discovered through the application of its DCE Platform® (deuterated chemical entity platform). Selective incorporation of deuterium into known molecules has the potential, on a case-by-case basis, to provide better pharmacokinetic or metabolic properties, thereby enhancing their clinical safety, tolerability or efficacy. Concert's lead product candidate CTP-543 is being developed for the treatment of alopecia areata, a serious autoimmune dermatological condition. Concert is also assessing a number of earlier-stage pipeline candidates. 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 regarding the development of CTP-543, the potential for CTP-543 to be a best-in-class treatment for the treatment of alopecia areata, the planned timing for filing an NDA for CTP-543 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, timing and design of future clinical trials, the availability and timing of data from ongoing and future clinical trials and the results of such trials, whether preliminary results, including safety profiles, 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 the timing of the submission of an NDA, the availability of regulatory approvals, the 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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