

Concert Pharmaceuticals Reports 2020 Financial Results and Provides Update on Clinical Programs

February 25, 2021

CTP-543 Phase 3 Program Advancing in Alopecia Areata; THRIVE-AA2, the Second CTP-543 Phase 3 Trial, to Initiate 1H2021

CTP-543 Long-Term Data Presented at EADV Show Hair Regrowth was Maintained or Improved in Patients with Alopecia Areata, Expanding Previous Phase 2 Results

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

LEXINGTON, Mass.--(BUSINESS WIRE)--Feb. 25, 2021-- [Concert Pharmaceuticals, Inc.](https://www.concertpharm.com) (NASDAQ: CNCE) today reported financial results for the year ended December 31, 2020, and provided an update on its product pipeline.

"As we enter 2021, Concert is now a Phase 3 company, moving our lead drug candidate, CTP-543, towards a new drug application and commercialization for the treatment of adults with moderate to severe alopecia areata. With our CTP-543 Phase 3 THRIVE-AA program advancing under Breakthrough Therapy designation, we believe that positive results from these trials would support submission of a new drug application in early 2023," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "We look forward to continuing the clinical development program for CTP-543, which we believe has the potential to become one of the first FDA-approved treatment options for adults impacted by alopecia areata."

Recent Business Highlights and Upcoming Milestones

CTP-543: An Investigational Treatment for Moderate to Severe Alopecia Areata

- **CTP-543 Maintains Hair Regrowth Beyond Initial 24-Week Treatment in Open Label, Long-Term Extension Study.** In the fourth quarter, the Company presented initial data from its ongoing open label, long-term extension study with CTP-543 in the late-breaking news session at the European Academy of Dermatology and Venereology (EADV) Virtual Congress. The presentation highlighted that in the open label extension study, treatment with CTP-543 showed that hair regrowth was maintained or improved, relative to the hair growth in the Company's previously conducted Phase 2 trials with 24-week treatment periods in patients with alopecia areata. The 8 mg and 12 mg twice-daily doses continued to be generally well tolerated, and adverse events were consistent with those reported in the Phase 2 trials.
- **CTP-543 THRIVE-AA1 Phase 3 Trial Enrollment Underway.** In November 2020, Concert began enrolling patients in the THRIVE-AA1 Phase 3 clinical trial. The Company expects to report topline results from the THRIVE-AA1 trial in 2022.
- **CTP-543 THRIVE-AA2 Phase 3 Trial Initiation Expected in the First Half of 2021.** A second Phase 3 trial of CTP-543, THRIVE-AA2, is expected to begin in the first half of 2021. Similar to the THRIVE-AA1 trial, THRIVE-AA2 will evaluate the regrowth of scalp hair after 24 weeks of dosing using the Severity of Alopecia Tool (SALT) in patients with moderate to severe alopecia areata.

Pipeline Updates

- **CTP-692 Phase 2 Results Reported.** In February 2021, Concert announced that its Phase 2 proof of concept clinical trial to evaluate CTP-692 as an adjunctive treatment for schizophrenia did not meet the primary or secondary endpoints. The Company does not intend to invest additional resources for the development of CTP-692.
- **Evaluation of Additional Pipeline Candidates Ongoing.** The Company is evaluating additional pipeline opportunities.

Full Year 2020 Financial Results

- **Cash and Investment Position.** Cash, cash equivalents and investments as of December 31, 2020 totaled \$130.0 million as compared to \$106.4 million as of December 31, 2019. In the first quarter of 2020, the Company closed a public offering of common stock and pre-funded warrants, raising net proceeds of \$70.1 million. In the fourth quarter of 2020, the Company raised net proceeds of \$22.5 million through its at-the-market offering program. Under its current operating plan, the Company expects its cash, cash equivalents and investments to fund the Company through 2021.
- **Revenue.** Revenue was \$7.9 million for the year ended December 31, 2020, compared to \$1.1 million for the year ended December 31, 2019. Revenue recognized in 2020 was primarily from the non-cash recognition of deferred revenue related to a previous agreement with Celgene Corporation.
- **R&D Expenses.** Research and development expenses were \$61.6 million for the year ended December 31, 2020, compared to \$59.8 million for the year ended December 31, 2019. The increase in R&D expenses in 2020 relates primarily

to costs associated with the Company's CTP-692 Phase 2 clinical trial in schizophrenia. R&D expenses are expected to increase in 2021 as the Company advances its CTP-543 Phase 3 THRIVE-AA clinical program in alopecia areata.

- **G&A Expenses.** General and administrative expenses were \$18.9 million for the year ended December 31, 2020, compared to \$20.3 million for the year ended December 31, 2019, a decrease of \$1.4 million primarily due to a decrease in legal expenses.
- **Net Loss.** For the year ended December 31, 2020, net loss attributable to stockholders was \$74.8 million, or \$2.40 per share, as compared to net loss attributable to stockholders of \$78.2 million, or \$3.29 per share, for the year ended December 31, 2019.

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 2020 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)

(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2020	2019	2020	2019
Revenue:				
License and research and development revenue	\$ 7	\$ 13	\$ 7,902	\$ 1,077
Total revenue	7	13	7,902	1,077
Operating expenses:				
Research and development	16,503	16,019	61,624	59,816
General and administrative	5,008	4,947	18,925	20,276
Total operating expenses	21,511	20,966	80,549	80,092
Loss from operations	(21,504)	(20,953)	(72,647)	(79,015)
Interest and other (expense) income, net	(886)	466	(2,204)	849
Loss before income taxes	(22,390)	(20,487)	(74,851)	(78,166)
Income tax benefit	—	—	85	—

Net loss					\$ (22,390)	\$ (20,487)	\$ (74,766)	\$ (78,166)
Net loss attributable to common stockholders - basic and diluted					(22,390)	(20,487)	(74,766)	(78,166)
Net loss per share applicable to common stockholders - basic and diluted					\$ (0.69)	\$ (0.86)	\$ (2.40)	\$ (3.29)
Weighted-average number of common shares used in net loss per share applicable to common stockholders - basic and diluted					32,666	23,848	31,200	23,740

Concert Pharmaceuticals, Inc.

Summary Balance Sheet Data

(in thousands)

(unaudited)

	December 31, 2020	December 31, 2019
Cash and cash equivalents	\$ 77,202	\$ 53,043
Investments, available for sale	52,766	53,395
Working capital	132,546	99,587
Total assets	159,263	137,471
Deferred revenue	2,750	10,533
Total stockholders' equity	131,162	101,457

About Concert

[Concert Pharmaceuticals](#) is a clinical stage biopharmaceutical company that is developing small molecule drugs that it discovered through the application of its [DCF 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](#) is in late-stage development 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 progress of clinical development of CTP-543, the timing of availability of clinical trial data, the timing of initiation and design of future clinical trials, the timing of regulatory filings 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 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, 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 Annual Report on Form 10-K or 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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