Concert Pharmaceuticals Reports 2021 Financial Results and Provides Company Update

March 3, 2022

Two Phase 3 Registrational Clinical Trials Fully Enrolled for CTP-543 for the Treatment of Alopecia Areata

CTP-543 THRIVE-AA1 Phase 3 Trial Topline Data Expected Second Quarter of 2022

CTP-543 THRIVE-AA2 Phase 3 Trial Topline Data Expected Third Quarter of 2022

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

LEXINGTON, Mass.--(BUSINESS WIRE)--Mar. 3, 2022-- Concert Pharmaceuticals, Inc. (NASDAQ: CNCE) today reported financial results for the year ended December 31, 2021.

"Our team is extremely proud and motivated by our success in enrolling more than 1,200 patients in our THRIVE-AA Phase 3 program in line with our projected timelines. This represents a significant milestone for Concert as we focus on commercializing CTP-543 to help meet the needs of the alopecia areata patient community," said Roger Tung, Ph.D., President and Chief Executive Officer of Concert Pharmaceuticals. "We expect 2022 to be a data rich year, with the first key CTP-543 Phase 3 data readout next quarter."

Recent Business Highlights and Upcoming Milestones

CTP-543: An Investigational Treatment in Phase 3 Trials for Moderate to Severe Alopecia Areata

- ***Topline Data in THRIVE-AA1 Expected in the Second Quarter of 2022.*** The Company expects to report topline results from the first CTP-543 Phase 3 trial, THRIVE-AA1, in the second quarter of 2022. THRIVE-AA1 is a randomized, double-blind, placebo-controlled Phase 3 clinical trial of CTP-543 to evaluate hair regrowth using the Severity of Alopecia Tool (SALT) after 24 weeks of dosing. The trial is evaluating 8 mg and 12 mg twice-daily doses of CTP-543 compared to placebo. The trial enrolled 708 adult patients with moderate to severe alopecia areata at sites in the U.S., Canada and Europe.

- ***Enrollment Completed in the THRIVE-AA2 Phase 3 Trial.*** Similar to THRIVE-AA1, THRIVE-AA2 is a randomized, double-blind, placebo-controlled Phase 3 clinical trial of CTP-543 to evaluate hair regrowth using SALT after 24 weeks of dosing. The trial is evaluating 8 mg and 12 mg twice-daily doses of CTP-543 compared to placebo. The trial enrolled 517 adult patients with moderate to severe alopecia areata at sites in the U.S., Canada and Europe. The Company expects to report topline results from the THRIVE-AA2 trial in the third quarter of 2022.

- ***New Drug Application (NDA) Filing Expected for Alopecia Areata in the First Half of 2023.*** If the CTP-543 clinical program is successful, the Company intends to file an NDA with the U.S. Food and Drug Administration (FDA) in the first half of 2023. The FDA has granted CTP-543 Breakthrough Therapy designation for the treatment of adult patients with moderate to severe alopecia areata and Fast Track designation for the treatment of alopecia areata. Alopecia areata is an autoimmune disease that may affect up to approximately 1.5 million Americans at any given time[1].

Partnered Program: AVP-786 for Neurological Disorders

- ***AVP-786 Late-Stage Trials to Complete in Third Quarter of 2022.*** Avanir Pharmaceuticals and Otsuka Pharmaceuticals are conducting a number of clinical trials evaluating AVP-786 in certain neurological disorders, including multiple Phase 3 studies in Alzheimer's agitation. Avanir and Otsuka have full responsibility for the development, reporting of clinical results and commercialization of AVP-786. Concert is entitled to potential future milestones and royalties. Two trials are expected to complete in the third quarter of 2022:

    - AVP-786 Phase 3 trial in Alzheimer's Agitation is projected to complete in July 2022.
    - AVP-786 Phase 2/3 trial in Negative Symptoms of Schizophrenia is projected to complete in August 2022.

Full Year 2021 Financial Results

- ***Cash and Investment Position.*** Cash, cash equivalents and investments as of December 31, 2021 totaled $141.6 million, compared to $130.0 million as of December 31, 2020. In November 2021, the Company raised gross proceeds of $65.0 million under a financing arrangement with BVF Partners L.P. and RA Capital Management, L.P. The financing consisted of the sale of common and preferred stock, warrants and a portion of Concert's right to receive potential future AVP-786 royalties under an existing licensing agreement with Avanir. Under its current operating plan, the Company expects its current cash and cash equivalents to fund the Company into the fourth quarter of 2022. In addition, Concert has the

potential to receive an additional $103.1 million upon the full exercise of the warrants issued in connection with the November 2021 financing.

- ***Revenue.*** Revenue was $32.6 million for the year ended December 31, 2021, compared to $7.9 million for the year ended December 31, 2020. Revenue recognized in 2021 was primarily attributable to the $32.0 million in cash proceeds received from Vertex Pharmaceuticals, Inc. for the purchase of potential future milestones under the companies' 2017 asset purchase agreement related to VX-561. Revenue recognized in 2020 was the result of the expiration of licensing options under a previous collaboration with Celgene Corporation.

- ***R&D Expenses.*** Research and development expenses were $87.6 million for the year ended December 31, 2021, compared to $61.6 million for the year ended December 31, 2020. The increase in research and development expenses relates primarily to the clinical development for CTP-543.

- ***G&A Expenses.*** General and administrative expenses were $22.5 million for the year ended December 31, 2021, compared to $18.9 million for the year ended December 31, 2020. The increase in general and administrative expenses relates primarily to increased external professional service expenses and non-cash stock-based compensation.

- ***Net Loss.*** For the year ended December 31, 2021, net loss attributable to common stockholders was $80.1 million, or $2.33 per share, compared to net loss applicable to common stockholders of $74.8 million, or $2.40 per share, for the year ended December 31, 2020.

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 financial results for the year ended December 31, 2021. 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 the financial results 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.

Consolidated Statements of Operations

(in thousands, except per share amounts)

(unaudited)

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2021	**2020**	**2021**	**2020**
Revenue:				
License and research and development revenue	$ 13	$ 7	$ 39	$ 7,902
Other revenue	—	—	32,539	—
Total revenue	13	7	32,578	7,902
Operating expenses:				

Research and development	26,995	16,503	87,555	61,624
General and administrative	5,970	5,008	22,531	18,925
Total operating expenses	32,965	21,511	110,086	80,549
Loss from operations	(32,952)	(21,504)	(77,508)	(72,647)
Investment income	2	102	46	1,202
Unrealized loss on marketable equity securities	(1,096)	(988)	(506)	(3,406)
Unrealized loss on warrant liabilities	(2,083)	—	(2,083)	—
Loss before income taxes	(36,129)	(22,390)	(80,051)	(74,851)
Income tax benefit	—	—	—	85
Net loss	$ (36,129)	$ (22,390)	$ (80,051)	$ (74,766)
Net loss per share attributable to common stockholders - basic and diluted	$ (1.01)	$ (0.69)	$ (2.33)	$ (2.40)
Weighted-average number of common shares used in net loss per share attributable to common stockholders - basic and diluted	35,646	32,666	34,405	31,200

Concert Pharmaceuticals, Inc.

Summary Balance Sheet Data

(in thousands)

(unaudited)

	December 31, 2021	December 31, 2020
Cash and cash equivalents	$ 141,636	$ 77,202
Investments, available for sale	—	52,766
Working capital	134,209	132,546
Total assets	165,316	159,263
Deferred revenue	7,595	2,750
Total stockholders' equity	112,225	131,162

About Concert

Concert Pharmaceuticals is a clinical stage 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 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 development of CTP-543, the timing of availability of clinical trial data, 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.

[1]Benigno M. Clinical, Cosmetic and Investigational Dermatology 2020.

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